Raptor Pharmaceutical Corp
Form 10-Q
May 07, 2015

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

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

For the quarterly period ended March 31, 2015

OR

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

Commission File Number: 000-25571

RAPTOR PHARMACEUTICAL CORP.
(Exact name of registrant as specified in its charter)

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

7 Hamilton Landing, Suite 100, Novato, CA 94949
(Address of Principal Executive Offices)

(415) 408-6200
(Registrant's telephone number)

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

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

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

Large accelerated filer Accelerated filer Non-accelerated filer Smaller reporting company

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

At May 5, 2015, there were 80,480,267 shares of the registrant's common stock outstanding.

RAPTOR PHARMACEUTICAL CORP.

FORM 10-Q

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PART I - FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS.

RAPTOR PHARMACEUTICAL CORP.
CONDENSED CONSOLIDATED BALANCE SHEETS
(Unaudited)
(In thousands, except shares and per share data)

	March 31, 2015	December 31, 2014
ASSETS		
Current assets:		
Cash and cash equivalents	$134,537	$149,613
Restricted cash	1,492	1,562
Accounts receivable	10,850	7,455
Inventories	4,317	9,134
Prepaid expenses and other	2,952	3,841
Total current assets	154,148	171,605
Noncurrent assets:		
Fixed assets, net	7,623	5,880
Goodwill	3,275	3,275
Intangible assets, net	2,914	2,974
Other assets	5,050	5,332
Total Assets	$173,010	$189,066
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$1,245	$2,550
Accrued liabilities	17,245	16,859
Common stock warrant liability	766	711
Note payable, current portion	12,000	9,000
Total current liabilities	31,256	29,120
Noncurrent liabilities:		
Note payable, net of current portion	48,000	51,000
Convertible notes	60,000	60,000
Total liabilities	139,256	140,120
Stockholders' equity:		
Preferred stock, $0.001 par value per share, 15,000,000 shares authorized, zero shares issued and outstanding	-	-
Common stock, $0.001 par value per share, 150,000,000 shares authorized, 69,394,765 and 68,861,366 shares issued and outstanding at March 31, 2015 and December 31, 2014, respectively	69	69
Additional paid-in capital	311,870	306,832
Accumulated other comprehensive loss	(607)	(60)
Accumulated deficit	(277,578)	(257,895)
Total stockholders' equity	33,754	48,946
Total Liabilities and Stockholders' Equity	$173,010	$189,066

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

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RAPTOR PHARMACEUTICAL CORP.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(Unaudited)
(In thousands, except shares and per share data)

	For the Three Months Ended March 31,	
	2015	2014
Revenues	$20,453	$12,134
Cost of sales	3,722	1,314
Gross profit	16,731	10,820
Operating expenses:		
Research and development	16,552	9,547
Selling, general and administrative	14,839	12,064
Total operating expenses	31,391	21,611
Loss from operations	$(14,660)	$(10,791)
Interest income	27	31
Interest expense	(4,498)	(2,979)
Foreign currency transaction (loss) gain	(476)	17
Adjustment to fair value of common stock warrants	(55)	(1,163)
Net loss before provision for income taxes	(19,662)	(14,885)
Provision for income taxes	(21)	(6)
Net Loss	$(19,683)	$(14,891)
Other comprehensive income (loss):		
Foreign currency translation gain (loss)	(547)	150
Comprehensive Loss	$(20,230)	$(14,741)
Net loss per share:		
Basic and diluted	$(0.28)	$(0.24)
Weighted-average shares outstanding:		
Basic and diluted	69,140,642	62,118,796

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

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RAPTOR PHARMACEUTICAL CORP.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(Unaudited)
(In thousands)

	For the Three Months Ended March 31,	
	2015	2014
Cash flows from operating activities:		
Net loss	$(19,683)	$(14,891)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	2,853	2,375
Fair value adjustment of common stock warrants	55	1,163
Amortization of intangible assets	60	60
Depreciation of fixed assets	302	117
Amortization of debt issuance cost	302	148
Changes in assets and liabilities:		
Accounts receivable	(3,395)	(898)
Inventories	4,817	(3,363)
Prepaid expenses and other assets	869	384
Accounts payable	(1,305)	(3,085)
Accrued liabilities	386	338
Deferred revenue	-	287
Net cash used in operating activities	(14,739)	(17,365)
Cash flows from investing activities:		
Net purchase of fixed assets	(2,045)	(187)
Change in restricted cash	70	(530)
Net cash used in investing activities	(1,975)	(717)
Cash flows from financing activities:		
Proceeds from the exercise of common stock warrants	-	1,826
Proceeds from the exercise of common stock options and ESPP	2,185	1,158
Offering costs	-	(29)
Net cash provided by financing activities	2,185	2,955
Effect of exchange rates on cash and cash equivalents	(547)	150
Net decrease in cash and cash equivalents	(15,076)	(14,977)
Cash and cash equivalents, beginning of period	149,613	83,052
Cash and Cash Equivalents, End of Period	$134,537	$68,075
Supplemental cash flow information:		
Interest paid	$2,869	$2,600
Income taxes paid	$120	$46
Supplemental disclosure of non-cash financing activities:		
Fair value of warrant liability reclassified to equity upon exercise	$-	$7,502

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

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RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
March 31, 2015
(Unaudited)

1. DESCRIPTION OF BUSINESS AND SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

The accompanying condensed consolidated financial statements reflect the financial position and results of operations of Raptor Pharmaceutical Corp. (the "Company" or "Raptor") and have been prepared in accordance with the accounting principles generally accepted in the United States of America ("GAAP") pursuant to the rules and regulations of the Securities and Exchange Commission (the "SEC"). Certain information and footnote disclosures have been condensed or omitted pursuant to such rules and regulations. The unaudited condensed consolidated financial statements have been prepared on the same basis as the annual financial statements and in the opinion of management reflect all adjustments, which include only normal recurring adjustments, necessary for a fair presentation of the periods presented. The condensed consolidated balance sheet as of December 31, 2014 has been derived from the Company’s audited financial statements as of such date but does not include all disclosures required by GAAP. This Form 10-Q should be read in conjunction with the audited financial statements and accompanying notes in the Company's Annual Report on Form 10-K for the year ended December 31, 2014.

Raptor is a biopharmaceutical company focused on developing and commercializing transformative treatments for people affected by rare and debilitating diseases. The Company's first product, PROCYSBI® (cysteamine bitartrate) delayed-release capsules ("PROCYSBI"), received marketing approval from the U.S. Food and Drug Administration ("FDA") on April 30, 2013 for the management of nephropathic cystinosis in adults and children six years and older. In Europe, PROCYSBI® gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate), received marketing authorization on September 6, 2013 from the European Commission ("EC"), for marketing in the European Union ("EU") as an orphan medicinal product for the management of proven nephropathic cystinosis. PROCYSBI received seven years of market exclusivity as an orphan drug in the United States and ten years of market exclusivity as an orphan drug in the EU. The Company commenced commercial sales of PROCYSBI in the United States in mid-June 2013, and in Europe in April 2014. For at least the near term, the Company's ability to generate revenues is entirely dependent upon sales of PROCYSBI in the United States for the management of nephropathic cystinosis in adults and children six years and older and in the EU for the management of proven nephropathic cystinosis.

Raptor's pipeline includes its proprietary delayed-release form of cysteamine, or RP103 and its proprietary oral 4-methylpyrazole, or Convivia®. Raptor currently has product candidates in clinical development designed to potentially treat Huntington's disease ("HD"), non-alcoholic steatohepatitis ("NASH") in children, Leigh syndrome and other mitochondrial disorders and aldehyde dehydrogenase deficiency ("ALDH2"). Raptor's preclinical programs are based upon bioengineered novel drug candidates that are designed to address current indications, target cancer and other diseases.

The Company is subject to a number of risks, including: the level of commercial sales of PROCYSBI in the United States and Europe; the ability to successfully launch PROCYSBI in other international markets; uncertainty whether the Company's research and development efforts will result in expanded labeling for PROCYSBI and additional commercialization for RP103 in various indications or additional commercial products; competition from other organizations; reliance on other entities for manufacturing; reliance on licensing the proprietary technology of others; uncertain patent protection; and the need to raise capital through equity and/or debt financings. Funding may not be available when needed if at all or on terms acceptable to the Company. If the Company exhausts its cash reserves and is unable to obtain adequate financing, it may be required to curtail planned operating expenditures, including its development programs.

Basis of Presentation

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

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March 31, 2015
(Unaudited)

Use of Estimates

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

Functional Currency

The Company's consolidated functional currency is the U.S. dollar. BV, SAS, and GMBH, the Company's Dutch subsidiary, French subsidiary, and German Subsidiary, respectively, use the European Euro as their functional currency. The CV subsidiary, a Cayman-based subsidiary, uses the dollar as its functional currency. At each quarter end, each foreign subsidiary's balance sheets are translated into U.S. dollars based upon the quarter-end exchange rate, while their statements of operations and comprehensive loss are translated into U.S. dollars based upon an average exchange rate during the period.

Segment Information

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

Fair Value of Financial Instruments

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

Cash and Cash Equivalents

The Company considers all highly liquid investments with original maturities of three months or less, when purchased, to be cash equivalents. The Company maintains cash and cash equivalents, which consist principally of money market funds, with high credit quality financial institutions. Such amounts exceed Federal Deposit Insurance Corporation insurance limits. Restricted cash represents certificates of deposit and compensating balances required by the Company's U.S. and European banks as collateral for credit cards and for access to a value-added tax deferral program. As of March 31, 2015, the Company had $134.5 million in cash and cash equivalents, of which $10.6 million was held by its foreign subsidiaries.

Revenue Recognition and Accounts Receivable

The Company recognizes revenue in accordance with the Financial Accounting Standards Board ("FASB") Accounting Standards Codification (“ASC”) 605, Revenue Recognition, when the following criteria have been met: persuasive evidence of an arrangement exists; delivery has occurred and risk of loss has passed; the seller's price to the buyer is fixed or determinable and collectability is reasonably assured. The Company determines that persuasive

evidence of an arrangement exists based on written contracts that define the terms of the arrangements. Pursuant to the contract terms, the Company determines when title to products and associated risk of loss has passed on to the customer. The Company assesses whether the fee is fixed or determinable based on the payment terms associated with the transaction and whether the sales price is subject to refund or adjustment. The Company assesses collectability based primarily on the customer's payment history and creditworthiness.

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PROCYSBI is currently available for U.S. distribution from the Company's U.S. specialty pharmacy partner, the Accredo Health Group, Inc. ("Accredo") which is currently the Company's only U.S. customer and ships directly to patients. The Company's distributor in the EU is the Almac Group, Ltd. PROCYSBI is not available in U.S. retail pharmacies. Authorization of coverage by patients' commercial insurance plans, Raptor's patient assistance program ("PAP") or government payors is a prerequisite to the shipment of PROCYSBI to U.S. patients. Prior to the third quarter of 2014, revenue was recognized in the United States once the product had been shipped by the specialty pharmacy to patients because the Company had not yet been able to reasonably estimate the third-party payor mix and resulting rebates based on its lack of sufficient historical data. Beginning July 2014, the Company was able to reasonably estimate and determine sales allowances; therefore the Company began recognizing PROCYSBI revenue at the point of sale to the specialty pharmacy. Revenue is currently recognized in the EU once confirmed orders from the pharmacies have been shipped and invoiced for payment by the distributor on the Company’s behalf.

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

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

Inventories and Cost of Sales

Inventories are stated at the lower of cost or market price, with cost determined on a first-in, first-out basis. Inventories are reviewed periodically to identify slow-moving inventory based on sales activity, both projected and historical, as well as product shelf-life. Prior to the approval of PROCYSBI by the FDA on April 30, 2013 and in Europe, prior to the approval by the EC on September 6, 2013, the Company recorded the purchase of raw materials and the manufacturing costs relating to PROCYSBI as research and development expense. Subsequent to FDA and EC approval, the Company began capitalizing these costs and manufacturing overhead as commercial inventory.

Products that have been approved by the FDA or other regulatory authorities are also used in clinical programs, to assess the safety and efficacy of the products for usage in diseases that have not been approved by the FDA or other regulatory authorities. The form of PROCYSBI utilized for both commercial and clinical programs is identical and, as a result, the inventory has an “alternative future use” as defined in authoritative guidance. Raw materials and purchased drug product associated with clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes and, therefore, does not have an “alternative future use”.

Upon launching PROCYSBI in June 2013 in the United States and April 2014 in the EU, the Company began recognizing cost of sales. Cost of sales includes the cost of inventory sold or reserved; manufacturing, manufacturing overhead and supply chain costs; product shipping and handling costs; and amortization of licensing approval milestone payments and licensing royalties payable to the University of California, San Diego ("UCSD").

Fixed Assets

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

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Goodwill and Intangible Assets

Intangible assets primarily include the intellectual property and other rights relating to DR Cysteamine (currently developed as RP103) and to an out-license acquired in a 2009 merger. The intangible assets related to RP103 are amortized using the straight-line method over the estimated useful life of 20 years, which is the life of the intellectual property patents. The 20-year estimated useful life is also based upon the typical development, approval, marketing and life cycle management timelines of pharmaceutical drug products.

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

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

Common Stock Warrant Liabilities

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

Note Payable

Note payable consists of a loan agreement with HealthCare Royalty Partners II, L.P. ("HC Royalty"), as lender, which was amended effective July 1, 2014. The amendment qualified as a modification of debt in accordance with ASC 470-50, Debt – Modifications and Extinguishments, as the Company determined it did not result in substantially different terms. The amended loan requires quarterly interest payments at an annual fixed interest rate of 8.0% of outstanding principal and includes a synthetic royalty component based on net product sales, including PROCYSBI, in a calendar year. The amended loan is a senior secured obligation of the Company.

Note payable is carried at its unpaid principal balance. The fixed and royalty interest under both agreements are recognized as interest expense as incurred.

Convertible Notes

Convertible notes include unsecured convertible senior notes and are carried at their unpaid principal balance. Interest on the notes is payable quarterly and the notes mature on August 1, 2019. If converted by a holder, upon conversion, the holder of the notes would receive shares of the Company’s common stock.

Debt Issuance Costs

Debt issuance costs are expenses associated with the issuance of the loan agreements with HC Royalty and the convertible notes. Debt issuance costs which were capitalized are being amortized over the life of the respective debt to interest expense using the interest method. Debt issuance costs are a component of Other Assets on the Company's consolidated balance sheets.

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Net Loss per Share

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

	Three Months Ended March 31,	
	2015	2014
Options to purchase common stock	9,663,956	9,584,383
Convertible debt	3,428,571	-
Warrants to purchase common stock	334,764	334,764
Restricted stock awards outstanding	212,980	-
Total Potentially Dilutive Securities	13,640,271	9,919,147

Comprehensive Loss

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

Stock-Based Compensation

Compensation costs related to the Company's stock incentive plans are measured at the grant date based on the fair value of the equity instruments awarded and are recognized over the period during which an employee is required to provide service in exchange for the award, or the requisite service period, which is usually the vesting period. The compensation expense for stock-based compensation awards is reduced by an estimate for forfeitures.

Incentive Plans

In May 2014, the Company’s shareholders approved the Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan (“Incentive Plan”) for executive officers, employees, and non-employee directors. The Incentive Plan provides for the grant of stock options, restricted stock units, and other types of awards to eligible participants. Long-term incentive awards granted under the Incentive Plan generally vest over a four-year period. Non-employee directors are also provided annual awards under the Incentive Plan that generally vest over a one year period. The cost of the awards is amortized over the vesting period on a straight-line basis.

In November 2014, the Company’s Board of Directors approved the 2014 Employment Commencement Stock Incentive Plan (“2014 Commencement Plan”) under Rule 5635(c)(4) of the Nasdaq Global Select Market for equity grants to induce new employees to enter into employment with the Company.

Employee Stock Purchase Plan

In July 2014, the Company’s shareholders approved the Raptor Pharmaceutical Corp. 2013 Employee Stock Purchase Plan (“ESPP”). Up to 1,000,000 shares may be issued pursuant to the ESPP. The purpose of the ESPP is to give the

Company’s employees an opportunity to acquire an equity interest in the Company through the purchase of shares of common stock at a discount. The ESPP allows eligible employees to purchase common stock at 85% of its fair value, subject to certain limits. Fair value as defined under the ESPP is the lesser of the closing market price of the common stock on the first day of the offering period or the last day of the offering period, which is a six-month period beginning on each May 15 and November 15.

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Research and Development Costs

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

Income Taxes

Income taxes are recorded under the liability method, under which deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to affect taxable income. Valuation allowances are established when necessary to reduce deferred tax assets to the amount expected to be realized. Based on the weight of available evidence, including cumulative losses since inception and expected future losses, the Company has determined that it is more likely than not that the deferred tax asset amount will not be realized and therefore a full valuation allowance has been provided on the Company's net deferred tax assets.

The Company identifies uncertain tax positions and discloses any potential tax liability on its financial statements. The Company recognizes interest and/or penalties related to income tax matters as a component of income tax expense. As of March 31, 2015, there were no accrued uncertain tax positions or interest and penalties related to uncertain tax positions.

The Company files U.S. Federal, California, various other state and other income tax returns and various foreign country income tax returns. The Company is currently not subject to any income tax examinations. Due to the Company's net operating losses ("NOLs"), generally all tax years remain open.

Reclassifications

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

Recent Accounting Pronouncements

In May 2014, the FASB issued Accounting Standards Update (“ASU”) 2014-09, Revenue from Contracts with Customers, which outlines a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The core principle of the revenue model is that “an entity recognizes revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services.” In applying the revenue model to contracts within its scope, the Company will: identify the contract(s) with a customer, identify the performance obligations in the contract, determine the transaction price, allocate the transaction price to the performance obligations in the contract, and recognize revenue when (or as) the entity satisfies a performance obligation. This ASU is effective for interim and annual periods beginning after December 15, 2016 and early adoption is not permitted. The Company does not believe the adoption of this ASU will have a material impact on its consolidated financial statements.

In June 2014, the FASB issued ASU 2014-12, Accounting for Share-Based Payments When the Terms of an Award Provide That a Performance Target Could Be Achieved after the Requisite Service Period. The ASU requires that a performance target that affects vesting, and that could be achieved after the requisite service period, be treated as a performance condition. A reporting entity should apply existing guidance in Topic 718 as it relates to awards with performance conditions that affect vesting to account for such awards. This ASU is effective for interim and annual periods beginning after December 15, 2015 and early adoption is permitted. The Company does not anticipate the adoption of this ASU will have a material impact on its consolidated financial statements.

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In August 2014, the FASB issued ASU 2014-15, Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern, which provides guidance on determining when and how reporting entities must disclose going-concern uncertainties in their financial statements. The ASU requires management to perform interim and annual assessments of an entity's ability to continue as a going concern within one year of the date of issuance of the entity's financial statements (or within one year after the date on which the financial statements are available to be issued, when applicable). Further, an entity must provide certain disclosures if there is "substantial doubt about the entity's ability to continue as a going concern." This ASU is effective for annual periods ending after December 15, 2016, and interim periods thereafter; early adoption is permitted.

In April 2015, the FASB issued ASU 2015-03, Interest - Imputation of Interest, which amends the presentation of debt issuance costs in the balance sheet as a direct deduction from the carrying amount of the related debt liability rather than as a deferred charge as presented under current guidance. ASU 2015-03 is effective for annual and interim periods beginning after December 15, 2015, and must be retrospectively applied. Early adoption is permitted. The Company does not expect the adoption of this amendment to have a material effect on its financial condition and results of operations.

2. FAIR VALUE MEASUREMENT

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

- Level 1 – Quoted market prices in active markets for identical assets or liabilities;

- Level 2 – Inputs other than level one inputs that are either directly or indirectly observable; and

- Level 3 – Unobservable inputs developed using estimates and assumptions, which are developed by the reporting entity and reflect those assumptions that a market participant would use.

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Determining which category an asset or liability falls within the hierarchy requires significant judgment. The Company evaluates its hierarchy disclosures each reporting period. The following table presents the assets and liabilities recorded that are reported at fair value on our consolidated balance sheets on a recurring basis.

Assets and Liabilities Measured at Fair Value on a Recurring Basis

(In thousands)

March 31, 2015	Level 1	Level 2	Level 3	Total
Assets				
Cash equivalents [1]	$115,205	$ -	$-	$115,205
Total	$115,205	$ -	$-	$115,205
Liabilities				
Common stock warrants	$-	$ -	$766	$766
Total	$-	$ -	$766	$766

December 31, 2014	Level 1	Level 2	Level 3	Total
Assets				
Cash equivalents [1]	$137,938	$ -	$-	$137,938
Total	$137,938	$ -	$-	$137,938
Liabilities				
Common stock warrants	$-	$ -	$711	$711
Total	$-	$ -	$711	$711

(1) Cash equivalents represent the fair value of the Company's investments in money market funds at March 31, 2015 and December 31, 2014.

Certain of the Company's common stock warrants are classified as liabilities and are, therefore, re-measured using the Black-Scholes option valuation model at the end of each reporting period with the change in value reported in the Company's consolidated statements of operations and comprehensive loss.

The following table presents a reconciliation of the Company's recurring fair value measurements categorized within Level 3 of the fair value hierarchy (liability-classified common stock warrants).

Changes in Level 3 Liabilities Measured at Fair Value on a Recurring Basis – Common Stock Warrants

	Three Months Ended March 31,	
(In thousands)	2015	2014
Beginning fair value	$711	$7,066
Change in fair value recognized in earnings	55	1,163

Exercises	-	(7,503)
Ending Fair Value	$766	$726

Effect of Raptor's Stock Price and Volatility Assumptions on the Calculation of Fair Value of Warrant Liabilities

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

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3. INVENTORIES

Inventories consist of raw materials, work-in-process and finished goods related to the manufacture of PROCYSBI. Raw materials include the active pharmaceutical ingredient ("API"), cysteamine bitartrate. Work-in-process includes third party manufacturing and associated labor costs relating to the Company's personnel directly involved in the production process. Also included in inventories are raw materials that may be used for clinical trials, which are charged to research and development (“R&D”) expense when consumed.

The following table summarizes the components of inventories.

(In thousands)	March 31, 2015	December 31, 2014
Raw materials	$588	$ 6,290
Work-in-process	2,892	721
Finished goods	837	2,123
Total Inventories	$4,317	$ 9,134

4. FIXED ASSETS

The following table presents the components of fixed assets and their estimated useful lives.

(In thousands)	March 31, 2015	December 31, 2014	Estimated useful lives
Assets under construction	$4,047	$ 2,393	-
Office furniture	2,198	2,198	7 years
Laboratory equipment	1,684	1,373	5 years
Computer hardware and software	895	815	3 years
Leasehold improvements	470	470	Lease term
Total at cost	9,294	7,249	
Less: accumulated depreciation	(1,671)	(1,369)	
Total Fixed Assets, Net	$7,623	$ 5,880	

Depreciation expense for the three months ended March 31, 2015 and 2014 was approximately $302 thousand and $117 thousand, respectively.

5. INTANGIBLE ASSETS AND GOODWILL

On December 14, 2007, the Company acquired the intellectual property and other rights to develop RP103 to treat various clinical indications from UCSD by way of a merger with Encode Pharmaceuticals, Inc., a privately held development stage company ("Encode"), which held the intellectual property license with UCSD. The fair value of the intangible assets at the time of acquisition was approximately $2.6 million.

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

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

A summary of intangibles acquired is as follows:

(In thousands)	Useful Life (Years)	March 31, 2015	December 31, 2014
Intangible asset (IP license for RP103) related to the Encode merger	20.0	$2,620	$ 2,620
Intangible assets (UCSD license - FDA and EC approval milestones)	20.0	1,250	1,250
Other intangible assets	16.0	240	240
Total intangible assets		4,110	4,110
Less accumulated amortization		(1,196)	(1,136)
Intangible Assets, Net		$2,914	$ 2,974

The intangible assets related to RP103 are being amortized over an estimated useful life of 20 years, which is the life of the intellectual property patents. The 20 year estimated useful life is also based upon the typical development, approval, marketing and life cycle management timelines of pharmaceutical drug products. Other intangible assets are being amortized using the straight-line method over an estimated useful life of 16 years, which is the life of the intellectual property patents.

During the three months ended March 31, 2015 and the year ended December 31, 2014, there was no intangible asset impairment recognized. During both the three months ended March 31, 2015 and 2014, the Company amortized approximately $60 thousand of intangible assets.

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

(In thousands)	Amortization Expense
2015 (remaining 9 months)	$ 178
2016	238
2017	238
2018	238
2019	238

The Company tested the carrying value of goodwill for impairment as of December 31, 2014 and determined that there was no impairment.

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6. ACCRUED LIABILITIES

Accrued liabilities consisted of:

(In thousands)	March 31, 2015	December 31, 2014
Personnel-related costs	$4,777	$ 6,879
Rebates and other sales deductions	2,687	3,231
Clinical trials and research and development costs	2,726	2,522
License royalty payable	1,123	972
Royalty-based interest payable	1,809	369
Manufacturing costs	1,120	284
Other	3,003	2,602
Total Accrued Liabilities	$17,245	$ 16,859

7. NOTE PAYABLE

On December 20, 2012, the Company entered into a loan agreement with HC Royalty, as lender, under which it agreed to borrow $50.0 million in two $25.0 million tranches. The Company received $23.4 million in net proceeds from the first tranche of the loan at closing in December 2012 and an additional $23.7 million in net proceeds in May 2013 from the second tranche upon FDA approval of PROCYSBI.

In July 2014, the Company entered into an amended and restated loan agreement with HC Royalty which revised the terms of the 2012 loan agreement between the Company and HC Royalty, and also provided for an additional $10 million in term loan funding. The interest rate was revised to an annual fixed rate of 8.0%, compared to the original interest rate of 10.75%. The loan also contains a synthetic royalty component based on net product revenues, including PROCYSBI, in a calendar year, and such royalty is payable quarterly. The variable royalty rate under the amended and restated loan agreement has been revised to 8.0% on the first $50 million of revenue and 2.0% on revenue in excess of $50 million. The first quarterly principal payment of $3 million is due in June 2015. All term loans under the amended and restated loan agreement mature on March 31, 2020. The loan and the Company's obligation to make payments thereunder shall terminate immediately when all payments received by HC Royalty equal $120.0 million.

Prior to July 1, 2014, with respect to the first $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bore a royalty rate of 6.25% of the first $25.0 million of product net revenues, 3.0% of product net revenues for such calendar year in excess of $25.0 million and up to $50.0 million, and 1.0% of product net revenues for such calendar year in excess of $50.0 million, payable quarterly. Prior to July 1, 2014, with respect to the second $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bore a royalty rate of 6.0% of the first $25.0 million of net product revenues for such calendar year, 3.0% of product net revenues for such calendar year in excess of $25.0 million and up to $50.0 million, and 1.0% of product net revenues for such calendar year in excess of $50.0 million, payable quarterly.

The Company's amended and restated loan agreement with HC Royalty includes affirmative and negative covenants, including the use of commercially reasonable efforts to exploit RP103 in specific markets and compliance with laws,

as well as restrictions on mergers and sales of assets, incurrence of liens, incurrence of indebtedness and transactions with affiliates and other requirements. To secure the performance of the Company's obligations under the loan, the Company granted a security interest to HC Royalty in substantially all of its assets, the assets of its domestic subsidiaries and a pledge of stock of certain of its domestic subsidiaries. The Company's failure to comply with the terms of the loan and related documents, the occurrence of a change of control of the Company or the occurrence of an uncured material adverse effect on the Company or the occurrence of certain other specified events, will result in an event of default under the loan that, if not cured or waived, could result in the acceleration of the payment of all of its indebtedness, as well as prepayment penalties, to HC Royalty and interest thereon. Under the terms of the security agreement, in an event of default, the lender can potentially take possession of, foreclose on, sell, assign or grant a license to use, the Company's pledged collateral and assign and transfer the pledged stock of certain of its subsidiaries.

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The Company received marketing approval of PROCYSBI from the FDA on April 30, 2013 and commenced shipment of PROCYSBI during June 2013, and as a result, royalties became payable to HC Royalty based upon net revenues of PROCYSBI. Interest expense on the loan, excluding amortization of debt issuance costs, for the three months ended March 31, 2015 and 2014 was approximately $3.0 million and $2.8 million, respectively.

The following table presents contractual principal payments of the note payable at March 31, 2015.

(In thousands)	Note Principal Payments
2015	$ 9,000
2016	12,000
2017	12,000
2018	12,000
2019	12,000
2020 and thereafter	3,000
Total	$ 60,000

Unamortized debt issuance costs on the loan agreement totaled $2.1 million and $2.3 million at March 31, 2015 and December 31, 2014, respectively. Amortization expense was $0.2 million and $0.1 million for the three months ended March 31, 2015 and 2014, respectively.

8. CONVERTIBLE NOTES

In July 2014, the Company sold $60 million aggregate principal amount of 8.0% convertible senior notes due 2019 to HC Royalty and other purchasers. These convertible notes require quarterly interest distributions at a fixed coupon rate equal to 8.0% until maturity or conversion, which will be no later than August 1, 2019. The convertible senior notes are convertible at the option of the holder at a conversion rate of 57.14 common shares per $1,000 principal amount of convertible senior notes at issuance (equivalent to a conversion price of $17.50 per common share), subject to adjustment in certain events. Upon conversion of these convertible senior notes by a holder, the holder will receive shares of the Company’s common stock.

In addition, the Company may elect to exercise the optional redemption, as defined in the note purchase agreement, in which case the convertible senior notes will convert into shares of common stock if the price of the common stock is at or above 175% of the applicable conversion price over a 30 consecutive day period. Upon the occurrence of a “change of control”, as defined in the note purchase agreement, the holders may require the Company to repurchase all or a portion of the notes for cash at 100% of the principal amount of the notes being purchased, plus a repayment premium and any accrued and unpaid interest. To secure the performance of the Company's obligations under the convertible notes agreement, the Company has assigned certain of its assets as collateral.

Interest expense on convertible notes, excluding amortization of debt issuance costs, was $1.2 million for the three months ended March 31, 2015. Unamortized debt issuance costs on these convertible notes totaled $2.7 million and $2.8 million at March 31, 2015 and December 31, 2014, respectively. Amortization expense for the three months ended March 31, 2015 was $0.1 million.

9. CAPITAL STRUCTURE

Preferred Stock

At March 31, 2015, the Company was authorized to issue 15,000,000 shares of $0.001 par value per share of preferred stock. There were no shares issued and outstanding.

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Common Stock

At March 31, 2015, the Company was authorized to issue 150,000,000 shares of $0.001 par value per share of common stock. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held. As of March 31, 2015 and December 31, 2014, there were 69,394,765 and 68,861,366 shares, respectively, of the Company's common stock issued and outstanding.

Stockholder Rights Plan

The Company’s stockholder rights plan entitles the holder of each outstanding share of common stock of the Company to one stock purchase right (a “Right”). Each Right entitles the registered holder to purchase from the Company one thousandth of a share of the Company’s Series A Participating Preferred Stock (the “Preferred Shares”) at a price of $15 per one one-thousandth of a Preferred Share (the “Purchase Price”), once the Rights become exercisable. The Rights will not be exercisable until the earlier of either (a) 10 days after the public announcement that a person, together with all affiliates or associates of such person, has become an “Acquiring Person” by obtaining beneficial ownership of 15% or more of the Company’s outstanding common stock, or (b) 10 business days (or a later date determined by the Board before any person or group becomes an Acquiring Person) after a person or group of affiliated or associated persons begins a tender or exchange offer which, if completed, would result in that person or group of affiliated or associated persons becoming an Acquiring Person. Each one one-thousandth of a share preferred stock, if issued, will have the same voting power as one one-hundred thirty-sixth (1/136th) of a share of common stock and will entitle holders to a per share payment equal to the payment made on one one-hundred thirty-sixth (1/136th) of a share of common stock, so that one full share of preferred stock would be entitled to receive a payment one one-hundred thirty-sixth (1/136th) of 1,000 times the per share payment to a share of common stock, provided that shares of the Company’s common stock are exchanged via merger, consolidation or a similar transaction. The Rights will expire on May 13, 2015 or on an earlier date if the Company redeems or exchanges the Rights.

Common Stock Issuance under At-The-Market ("ATM") Agreement

On April 30, 2012, the Company entered into an "At-the-Market" ("ATM") Sales Agreement, with Cowen and Company, LLC ("Cowen"), under which the Company could, at its discretion, sell its common stock with a sales value of up to a maximum of $40.0 million through ATM offerings on the NASDAQ Stock Market. On July 3, 2013, the Company and Cowen amended and restated the Sales Agreement (the "Amended and Restated Sales Agreement") to increase the aggregate gross sales proceeds that could be raised to $100 million. Cowen was the sole sales agent for any sales made under the ATM for a 3.0% commission on gross proceeds. The common stock was sold at prevailing market prices at the time of the sale of common stock, and, as a result, prices varied. During the three months ended March 31, 2014, there were no shares sold under the ATM. As of December 31, 2014, the Company did not have any remaining shares available under the ATM for future sales of the Company's common stock.

Common Stock Warrants

During the three months ended March 31, 2015, there were no warrants exercised. During the three months ended March 31, 2014, the Company received approximately $1.8 million from the exercise of warrants in exchange for the issuance of 611,606 shares of the Company's common stock.

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The number of common stock warrants outstanding as of March 31, 2015 were as follows:

	Number of Shares	Exercise Price	Expiration Date
Issued in connection with Encode merger	233,309	$2.87	12/13/2015
TorreyPines warrants assumed in 2009 Merger	3,503	157.08	9/26/2015
Issued to placement agent in Aug. 2010	97,952	3.08	8/12/2015
Total Warrants Outstanding	334,764	$4.54 (1)	

(1) Weighted average exercise price

The warrants issued by the Company in the August 2010 private placement and the December 2009 equity financing contain a conditional obligation that may require the Company to transfer assets to repurchase the warrants upon the occurrence of potential future events. Under ASC 480, a financial instrument that may require the issuer to settle the obligation by transferring assets is classified as a liability. Therefore, the Company has classified the warrants from both financings as liabilities and marks them to fair value at each period end. All warrants issued in connection with the December 2009 equity financing have been exercised or expired as of December 31, 2014.

A Black-Scholes option-pricing model was used to obtain the fair value of the warrant liabilities using the following assumptions at March 31, 2015 and December 31, 2014:

	August 2010 Private Placement Investors and Placement Agent	
	March 31, 2015	December 31, 2014
Fair value (in thousands)	$766	$ 711
Black-Scholes inputs:		
Stock price	$10.87	$ 10.28
Exercise price	$3.08	$ 3.08
Risk free interest rate	0.12 %	0.12 %
Volatility	95.00%	95.00 %
Expected term (years)	0.42	0.50
Dividend	-	-

10. STOCK-BASED COMPENSATION

2010 Stock Incentive Plan

The Company's 2010 Stock Incentive Plan, as amended, provides for stock options, restricted shares or restricted share units to be granted to its employees, independent contractors, consultants or directors. On November 25, 2014, as a key requirement of the Company's strategy of strengthening its leadership team and employee base, continuing the

expansion of its commercial activities into new territories, and increasing the expansion of its product development programs, the Company's Board of Directors approved the Raptor Pharmaceutical Corp. 2014 Employment Commencement Stock Incentive Plan. The plan was approved pursuant to Rule 5635(c)(4) of the Nasdaq Global Select Market for equity grants to induce new employees to enter into employment with the Company. Up to 2,400,000 shares may be issued under this plan.

During the three months ended March 31, 2015 and 2014, the Company received approximately $2.2 million and $1.2 million, respectively, from the exercise of stock options. At March 31, 2015, there were 928,637 and 1,415,131 shares remaining available for issuance under the 2010 Stock Incentive Plan and 2014 Employment Commencement Stock Incentive Plan, respectively.

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

	Three Months Ended March 31,	
(In thousands)	2015	2014
Cost of goods sold	$32	$40
Research and development	584	559
Sellin, general and administrative	2,237	1,776
Total Stock-Based Compensation Expense	$2,853	$2,375

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

	Option	Weighted-average
Three Months Ended March 31, 2015	Shares	Exercise Price
Beginning balance	8,857,961	$ 7.71
Granted	1,635,861	9.75
Exercised	(533,399)	4.10
Canceled	(296,467)	11.03
Outstanding Balance at Period End	9,663,956	8.15

Restricted Stock Units

At March 31, 2015, there were 212,980 RSUs outstanding, of which none had vested. There were 214,210 RSUs granted and 1,230 RSUs forfeited related to employee departures during the three months ended March 31, 2015. During the three months ended March 31, 2015, there were no RSU distributions. Unvested RSUs at March 31, 2015 vest through 2019.

Employee Stock Purchase Plan

The ESPP allows a maximum of 1,000,000 shares of common stock to be purchased in aggregate for all employees. At March 31, 2015, 21,280 shares had been purchased, and there remained a negligible amount of uninvested employee contributions in the ESPP. As of March 31, 2015, there were approximately 978,720 shares reserved for future issuance under the ESPP.

11. COMMITMENTS AND CONTINGENCIES

The Company maintains several contracts with drug labelers and distributors, research organizations, contract manufacturers, clinical organizations and clinical sites, primarily to assist with clinical research and clinical and commercial manufacturing and distribution of PROCYSBI and clinical manufacturing of drug product for the Company's HD and NASH clinical collaborations. The Company's contractual obligations did not change significantly during the three months ended March 31, 2015 compared to those disclosed as of December 31, 2014.

12. SUBSEQUENT EVENTS

On April 2, 2015 the Company completed a public offering of 10.925 million shares of its common stock for net proceeds after expenses of approximately $92 million.

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ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion in conjunction with our unaudited condensed consolidated financial statements as of March 31, 2015, and the notes to such unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q. All references to the “Company," "we," "our" and "us" include the activities of Raptor Pharmaceutical Corp., Raptor Pharmaceuticals Inc., Raptor European Products, LLC, RPTP European Holdings C.V., Raptor Pharmaceuticals Europe B.V., Raptor Pharmaceuticals France SAS and Raptor Pharmaceuticals Germany GmbH.

This Quarterly Report on Form 10-Q, including this "Management's Discussion and Analysis of Financial Condition and Results of Operations" section, 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," "predicts," "intends," "continues," "estimates," "potential," "opportunity" or the negative of these terms or other comparable terminology. All such statements, other than statements of historical facts, including our financial condition, future results of operations, projected revenues and expenses, business strategies, operating efficiencies or synergies, competitive positions, growth opportunities for existing intellectual properties, technologies, products, plans, and objectives of management, markets for our securities, and other matters, involve substantial risks and uncertainties and constitute forward-looking statements for the purpose of the safe harbor provided by Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Such forward-looking statements, wherever they occur, are necessarily estimates reflecting the best judgment of our senior management on the date on which they were made, or if no date is stated, as of the date of the filing made with the SEC in which such statements were made. You should not place undue reliance on these statements, which only reflect information available as of the date that they were made. We cannot give you any assurance that such forward-looking statements will prove to be accurate and such forward-looking events may not occur. Our business's actual operations, performance, development and results might differ materially from any forward-looking statement due to known and unknown risks, uncertainties, assumptions and contingencies, including those described in the section titled "Risk Factors" in Part II, Item 1A of this Quarterly Report on Form 10-Q. Unless required by U.S. federal securities laws and the rules and regulations of the SEC, we do not undertake any obligation and disclaim any intention to update or release publicly any revisions to these forward-looking statements after the filing of this Quarterly Report on Form 10-Q to reflect later events or circumstances or to reflect the occurrence of unanticipated events or for any other reason.

Plan of Operation and Overview

We are a biopharmaceutical company focused on developing and commercializing transformative treatments for people affected by rare and debilitating diseases.

Our product, PROCYSBI, received marketing approval from the FDA in April 2013 for the management of nephropathic cystinosis in adults and children six years and older. In Europe, PROCYSBI gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate), received a marketing authorization in September 2013 from the EC as an orphan medicinal product for the management of proven nephropathic cystinosis in the EU. The EU marketing authorization allows us to commercialize PROCYSBI in the 28 Member States of the EU plus Norway, Liechtenstein and Iceland (which are not EU Member States but are part of the European Economic Area, or EEA). PROCYSBI received seven and ten years of market exclusivity due to its designation as an orphan drug in the United States and the EU, respectively. We achieved first commercial sales of PROCYSBI in the United States in June 2013 and in the EU, specifically in Germany, in April 2014.

Clinical Development Programs

Our three active clinical development programs utilize RP103, which contains the same active pharmaceutical ingredient as PROCYSBI, cysteamine bitartrate. RP103 is our proprietary capsule formulation containing delayed-release enteric coated microbeads of cysteamine bitartrate. Cysteamine bitartrate was approved in the United States in 1994 and the EU in 1997 as an orally available immediate-release powder in a capsule for the management of cystinosis. We have an exclusive worldwide license from the University of California, San Diego ("UCSD"), to delayed-release cysteamine bitartrate, which is the basis for our proprietary formulation of cysteamine. We currently have product candidates in clinical development designed to potentially treat HD, non-alcoholic steatohepatitis ("NASH") in children, and Leigh syndrome and other mitochondrial disorders.

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

Preclinical Product Candidates

Our preclinical programs, for which we may seek development partners in the future, include our cysteamine dioxygenase, or ADO, program.

Future Activities

Over the remainder of the fiscal year, our efforts will be focused on increasing sales of PROCYSBI in the U.S. and Europe; launching or providing access to PROCYSBI in other countries in the EU and other select countries around the world; maintain progress on filing a New Drug Submission, or NDS, for cysteamine bitartrate delayed-release capsules with Health Canada; conducting a clinical trial to evaluate PROCYSBI in cystinosis patients that are cysteamine-naïve, as well as other supporting trials in underdeveloped markets; developing select global markets with significant numbers of known cystinosis patients; screening for undiagnosed and unidentified adult nephropathic cystinosis patients; supporting regulatory pathways and/or clinical trials of RP103 for the potential treatment of HD, pediatric NASH, Leigh syndrome and mitochondrial disorders; enhancing and expanding our product manufacturing capabilities; preparing for potential clinical studies of RP103 in new therapeutic indications; supporting our novel preclinical programs; seeking additional business development partners in Asia for our Convivia product candidate; and developing new preclinical, clinical and or commercial opportunities, including proprietary molecules or development candidates discovered in-house and in-licensed and acquired technologies.

Results of Operations – Three Months Ended March 31, 2015 and 2014

Revenue

The first U.S. sales of PROCYSBI commenced in June 2013. The first European sales of PROCYSBI commenced in April 2014. For the three months ended March 31, 2015 and 2014, we recognized $20.5 million and $12.1 million, respectively, in PROCYSBI net product sales for each region.

Prior to the third quarter of 2014, revenue from the sale of PROCYSBI was recognized based on the amount of product sold through to the patients. Beginning July 2014, we were able to reasonably estimate and determine sales allowances; therefore we began recognizing PROCYSBI revenue at the point of sale to the specialty pharmacy, which resulted in the one-time non-recurring recognition of an additional $4.4 million in net revenues during the three months ended September 30, 2014.

Cost of Sales

Cost of sales for the three months ended March 31, 2015 and 2014 were $3.7 million and $1.3 million, respectively, which was primarily attributable to an increase in inventory reserves as well as an increase in royalty fees on net product sales. Cost of sales primarily includes: raw materials and manufacturing costs for our commercial product PROCYSBI, amortization of licensing milestone payments, royalty fees due to UCSD on our net product sales, other indirect costs such as distribution, labeling, shipping and supplies, and provision for inventory expiration. Costs capitalized as inventory are expensed as cost of sales as product is sold.

Prior to FDA approval of PROCYSBI, our commercial manufacturing costs had been recorded as research and development expenses. As a result, our cost of sales in 2014 reflected a lower average direct product cost per unit will be recorded in the future.

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Research and Development

Research and development expenses include medical, clinical, regulatory, quality (excluding manufacturing quality control expenses), pharmacovigilance and research salaries and benefits; expenses associated with the manufacturing and testing of PROCYSBI inventory for our commercial launch in the United States and in Europe which were expensed prior to drug approvals; preclinical studies; clinical trials; regulatory and clinical consultants; research supplies and materials; amortization of intangible assets and allocated human resources and facilities expenses.

Research and development expenses increased approximately 73% to $16.6 million for the three months ended March 31, 2015 from $9.5 million during the three months ended March 31, 2014. The $7.0 million increase was primarily due to raw materials charged to research and development that was determined would be used for process and manufacturing development during the remainder of the year, as well as expansion of medical affairs activities to support projects and programs. The following table shows major program expenses recorded as research and development expenses.

Detail of Research and Development Expenses

	Three Months Ended March 31,	
(In millions)	2015	2014
RP103:		
Cystinosis (pre-commercial and extension)	$3.8	$3.7
HD (clinical)	0.5	0.5
NASH (clinical)	0.7	0.4
Preclinical programs	0.5	0.6
Other programs	0.7	0.6
Process and manufacturing development	4.6	-
R&D personnel and other costs not allocated to programs	5.8	3.7
Total Research and Development Expenses	$16.6	$9.5

Selling, General and Administrative Expenses

Selling, general and administrative expenses primarily include commercial expenses related to marketing and sales operations in the U.S. and EU, including marketing and pricing studies, advertising, sales force commissions and other expenses, and market access support activities; commercial launch expenses for PROCYSBI, including patient support activities such as reimbursement assistance and establishing a customer relationship management system for our PROCYSBI sales team, and salaries and benefits for our commercial operations; intellectual property, legal and audit fees, finance, executive expenses; and other administrative and facilities costs.

Selling, general and administrative expenses increased approximately 23% to $14.8 million for the three months ended March 31, 2015 from $12.1 million in the three months ended March 31, 2014. The $2.7 million increase was primarily due to increased staffing and promotional support for commercial operations of PROCYSBI in the U.S. and building the support staff for our European operations.

Interest Expense

Interest expense for the three months ended March 31, 2015 and 2014 was $4.5 million and $3.0 million, respectively. The increase in interest expense was due primarily to the interest increase the debt principal outstanding as a result of

the issuance of $60 million of convertible notes in July 2014 and an amendment to the $50.0 million loan agreement that we entered into with HealthCare Royalty Partners II, L.P., or HC Royalty, in December 2012, which was amended in July 2014 to provide for an additional $10 million in term loan funding. Also contributing to the increase in interest expense was a small increase in royalty interest fees payable to HC Royalty.

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Adjustment to the Fair Value of Common Stock Warrants

The adjustment to the fair value of common stock warrants was a loss of $55 thousand and $1.2 million for the three months ended March 31, 2015 and 2014, respectively. The loss for the three months ended March 31, 2015 was due primarily to an increase in stock price in the first quarter of 2015. The loss for the three months ended March 31, 2014 was due primarily to an increase in our stock price in the first quarter of 2014. At March 31, 2015 and March 31, 2014, the remaining warrants outstanding were 334,764. All warrants will expire in 2015.

Application of Critical Accounting Policies

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

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

Revenue Recognition and Accounts Receivable

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

PROCYSBI is currently available for U.S. distribution from our U.S. specialty pharmacy partner, the Accredo Health Group, Inc. (“Accredo”) which is currently our only U.S. customer and ships directly to patients. Our commercial launch in the EU commenced in April 2014, with the Almac Group, Ltd. as our distributor. PROCYSBI is not available in U.S. retail pharmacies. Prior to the third quarter of 2014, revenue was recognized in the United States once the product had been shipped by the specialty pharmacy to patients because we had not yet been able to reasonably estimate the third-party payor mix and resulting rebates due to the lack of sufficient historical data. Beginning July 2014, we were able to reasonably estimate and determine sales allowances; therefore we began recognizing PROCYSBI revenue at the point of sale to the specialty pharmacy. Revenue is currently recognized in the EU once confirmed orders from the pharmacies have been shipped and invoiced for payment by our distributor on our behalf.

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

such allowances in the quarter the changes are known.

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

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Inventories and Cost of Sales

Inventories are stated at the lower of cost or market price, with cost determined on a first-in, first-out basis. Inventories are reviewed periodically to identify slow-moving inventory based on sales activity, both projected and historical, as well as product shelf-life. Prior to the approval of PROCYSBI by the FDA on April 30, 2013 and in Europe, prior to EC approval on September 6, 2013, we recorded the purchase of raw materials and the manufacturing costs relating to PROCYSBI as research and development expense. Subsequent to FDA and EC approval, we began capitalizing these costs and manufacturing overhead as commercial inventory. Upon launching PROCYSBI in mid-June 2013 in the United States and April 2014 in the EU, we began recognizing cost of sales. Cost of sales includes the cost of inventory sold or reserved; manufacturing, manufacturing overhead and supply chain costs; product shipping and handling costs; and amortization of licensing approval milestone payments and licensing royalties payable to UCSD.

Note Payable

Note payable consists of loan agreements with HC Royalty as lender, totaling $60 million. In July 2014, we modified our original December 2012 loan agreement with HC Royalty as lender, under which we borrowed $50 million in two $25 million tranches received in December 2012 and May 2013, to provide for an additional $10 million in term loan funding. The interest rate was revised to an annual fixed rate of 8.0%, compared to the original interest rate of 10.75%. The loan also contains a synthetic royalty component based on net product revenues, including PROCYSBI, in a calendar year, and such royalty is payable quarterly. The variable royalty rate under the amended and restated loan agreement has been revised to 8.0% on the first $50 million of revenue and 2.0% on revenue in excess of $50 million. The first loan principal payment of $3 million per quarter is due in June 2015 and principal payments continue at that rate for the term of the loan. All term loans under the amended and restated loan agreement mature on March 31, 2020. The loan and our obligation to make payments thereunder shall terminate immediately when all payments received by HC Royalty equal $120 million.

Prior to July 1 2014, the loan bore interest at an annual fixed rate of 10.75% of outstanding principal and included a synthetic royalty component based on net product sales, including PROCYSBI, in a calendar year. With respect to the first $25 million tranche, for each calendar year, the loan bore a royalty rate of 6.25% of the first $25 million of product net revenues, 3.0% of product net revenues for such calendar year in excess of $25 million and up to $50 million, and 1.0% of product net revenues for such calendar year in excess of $50 million, payable quarterly. With respect to the second $25 million tranche, for each calendar year, the loan bore a royalty rate of 6.0% of the first $25 million of product net revenues for such calendar year, 3.0% of product net revenues for such calendar year in excess of $25 million and up to $50 million, and 1.0% of product net revenues for such calendar year in excess of $50 million, payable quarterly.

The fixed and royalty interest are recognized as interest expense as incurred. The revenue related royalty interest may lead to significant fluctuations in interest expense from period to period due in part to the tiered royalty rate in effect within a calendar year.

Convertible Notes

In July 2014, we sold $60 million aggregate principal amount of 8.0% convertible senior notes due 2019 to HC Royalty and other purchasers. These convertible notes require quarterly interest payments at a fixed coupon rate equal of 8.0% until maturity or conversion, which will be no later than August 1, 2019. The convertible senior notes are convertible at the option of the holder at a conversion rate of 57.14 common shares per $1,000 principal amount of convertible senior notes at issuance (equivalent to a conversion price of $17.50 per common share), subject to adjustment in certain events. In addition, the convertible senior notes will automatically convert into shares of common stock if the price of the common stock is at or above 175% of the applicable conversion price over a 30

consecutive day period. Upon conversion of these convertible senior notes by a holder, the holder will receive shares of our common stock. The fixed interest payments are recognized as interest expense as incurred.

Impairment of Goodwill and Intangible Assets

Goodwill represents the excess of the purchase price over the fair value of tangible and identified intangible net assets of businesses acquired. Goodwill is not amortized, but is evaluated for impairment on an annual basis or more often when impairment indicators are present. We have one reporting unit. Therefore, our consolidated net assets, including existing goodwill and other intangible assets, are considered to be the carrying value of the reporting unit. If the carrying value of the reporting unit is in excess of its fair value, an impairment may exist, and we must perform the second step of the analysis, in which the implied fair value of the goodwill is compared to its carrying value to determine the impairment charge, if any. If the estimated fair value of the reporting unit exceeds the carrying value of the reporting unit, goodwill is not impaired and no further analysis is required. We performed our goodwill impairment test as of December 31, 2014 and noted no impairment.

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

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

Common Stock Warrant Liabilities

The common stock warrants we issued in connection with certain fiscal year 2010 equity financings contain conditional obligations that may require us to transfer cash to settle the warrants upon the occurrence of certain fundamental transactions. Therefore, we have classified the warrants as liabilities. We re-measure the liability at the end of every reporting period with the change in value reported in our consolidated statements of operations. At the exercise date, the fair values of these warrants are re-measured and reclassified to equity.

We use the Black-Scholes option pricing model as our method of valuation for warrants that are subject to warrant liability accounting. The determination of the fair value as of the reporting date is affected by our stock price as well as assumptions regarding a number of highly complex and subjective variables. These variables include, but are not limited to, expected stock price volatility over the term of the security and risk-free interest rate. In addition, the Black-Scholes option pricing model requires the input of an expected life for the securities, which is based on the contractual terms of the underlying agreement. The fair value of the warrant liability is revalued each balance sheet date utilizing Black-Scholes valuation model computations with the decrease or increase in fair value being reported in the statement of operations as other income or expense, respectively. The primary factors affecting the fair value of the warrant liability are our stock price and volatility.

We reported a net loss of $19.7 million for the three months ended March 31, 2015. If our March 31, 2015 closing stock price had been 10% lower, our net loss would have been approximately $0.1 million lower. If our March 31, 2015 closing stock price had been 10% higher, our net loss would have been approximately $0.1 million higher.

A 10% increase or decrease of our volatility assumption for warrants at March 31, 2105 would not have had a material effect on our net loss due to the low number of warrants that remain outstanding at March 31, 2015.

Stock-Based Compensation

Determining the fair value of share-based awards at the grant date requires judgment, including estimating future stock price volatility and employee stock option exercise behavior.

We based our Black-Scholes inputs on the following factors: the expected life of six years was based upon our assessment of the ten-year term of the stock options issued, along with the fact that we have been a commercial company since June 2013 and as a result, more option holders have been exercising stock options; the risk-free interest rate was based on current constant maturity treasury bill rates for six years; the volatility was based on a combination of the actual annualized volatility of our common stock price as quoted on NASDAQ since the closing of our merger with Raptor Pharmaceuticals Corp. on September 30, 2009 and of annualized volatility of peer companies; the forfeiture rate was based on our assessment of our historical employee turnover; and the dividend rate was based on our current decision to not pay dividends on our stock at our current corporate stage of development. If actual results differ significantly from these estimates, stock-based compensation expense and results of operations could be materially impacted. See Note 10 of our consolidated financial statements for a further discussion of our accounting

for stock-based compensation.

Income Taxes

Income taxes are recorded under the liability method, under which deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to affect taxable income. Valuation allowances are established when necessary to reduce deferred tax assets to the amount expected to be realized. Based on the weight of available evidence, including cumulative losses since inception and expected future losses, we have determined that it is more likely than not that the deferred tax asset amount will not be realized and therefore a full valuation allowance has been provided on our net deferred tax assets. We intend to maintain the valuation allowance until sufficient positive evidence exists to support the reversal of the valuation allowance. Any decision to reverse part or all of the valuation allowance would be based on our estimate of future profitability.

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We identify uncertain tax positions and record or disclose any resulting potential tax liability based upon whether the position is more likely than not sustainable upon examination. We consider proposed assessments by tax authorities, changes in facts and circumstances, issuance of new regulations or new case law and negotiations between tax authorities of different countries concerning our transfer prices or intellectual property transfers. As of March 31, 2015, we had identified no uncertain tax positions.

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

Liquidity and Capital Resources

Capital Resource Requirements

As of March 31, 2015, we had approximately $134.5 million in cash and cash equivalents, approximately $31.3 million in current liabilities (of which approximately $0.8 million represented the common stock warrant liability, which is expected to be settled in shares) and approximately $122.5 million of net working capital. In 2015, we will commence principal payments on notes payable of $3 million per quarter.

In April 2015, we completed a public offering of 10.925 million shares of our common stock for net proceeds of $92 million. Our cash and cash equivalents are expected to be sufficient to fund operations through at least the second half of 2017, based on current operating plan assumptions.

In July 2014, we arranged a $70 million funding from HealthCare Royalty Partners (“HC Royalty”) and its affiliates. The funding included $60 million in new convertible senior notes and an additional $10 million of funding pursuant to an amended and restated loan agreement.

On April 30, 2012, we entered into a sales agreement with Cowen and Company, or Cowen, to sell shares of our common stock, with aggregate gross sales proceeds of up to $40 million, from time to time through an "at the market," or ATM, equity offering program under which Cowen acts as sales agent. We paid a 3% commission to Cowen on any sales pursuant to this sales agreement. On July 3, 2013, we amended and restated the sales agreement to increase the aggregate gross sales proceeds that could be raised to $100 million. All shares then available under the gross sales proceeds authorization had been sold under the ATM agreement as of December 31, 2014.

Future Funding Requirements

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

- The continuing sales of PROCYSBI in the United States and Europe;
- The ongoing costs of establishing and maintaining sales and marketing capabilities in the United States, Europe and other countries;
- Our ability to negotiate reimbursement and pricing of PROCYSBI in various countries outside of the United States;
- The cost of our manufacturing-related activities in support of PROCYSBI and RP103;
- The cost of activities and outcomes related to the regulatory submission of cysteamine bitartrate delayed-release capsules in Canada;
- The cost of additional clinical trials in order to obtain regulatory approvals for PROCYSBI in non-U.S. and non-European countries;

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- The timing and cost of our ongoing clinical programs for RP103, including: evaluating PROCYSBI in treatment-naïve cystinosis patients, and other supportive studies; evaluating RP103 as a potential treatment for HD; evaluating RP103 as a potential treatment for NASH in children; evaluating RP103 as a potential treatment for Leigh syndrome and other mitochondrial disorders;
- The cost of regulatory submissions, as well as the preparation, initiation and execution of clinical trials in potential new clinical indications using RP103;
- The cost of evaluating and potentially acquiring or in-licensing new drug compound(s) for potential clinical development and commercialization; and
- The cost of filing, continuing surveillance, prosecuting, defending and enforcing existing or new patent claims.

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

Commitments and Contingencies

We maintain several contracts with drug labelers and distributors, research organizations, contract manufacturers, clinical organizations and clinical sites, primarily to assist with clinical research, clinical and commercial manufacturing of PROCYSBI and clinical manufacturing for our HD and our NASH clinical collaborations and our clinical study of RP103 in Leigh syndrome and other mitochondrial disorders. Our contractual obligations have not materially changed during the three months ended March 31, 2015 compared to those discussed in our Annual Report on Form 10-K for the year ended December 31, 2014, filed with the SEC on March 2, 2015.

Off-Balance Sheet Arrangements

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

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our market risks during the three months ended March 31, 2015 have not materially changed from those discussed in our Annual Report on Form 10-K for the year ended December 31, 2014, filed with the SEC on March 2, 2015.

ITEM 4. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Disclosure controls and procedures are controls and other procedures that are designed to ensure that information required to be disclosed in our reports filed or submitted under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed in our reports filed under the Exchange Act is accumulated and communicated to management, including our principal executive officer and our principal financial officer, as appropriate, to allow timely decisions regarding required disclosure. As previously disclosed in Part II, Item 9A of our Annual Report on Form 10-K for the year ended December 31, 2014, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were not effective as of December 31, 2014 due to a material weakness in our internal control over financial reporting related to our inventory costing and overhead allocations for our commercial product PROCYSBI. Our management, with the participation of our principal executive officer and principal financial officer, evaluated, as of the end of the period covered by this Quarterly Report on Form 10-Q, the effectiveness of our disclosure controls and procedures. Based on their evaluation at the end of the period covered by this Quarterly Report

on Form 10-Q, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were not effective as of the end of such period because the remediation activities designed to address the root causes of the material weakness identified in 2014, as described in Part II, Item 9A of our Annual Report on Form 10-K for the year ended December 31, 2014, had not operated for a sufficient time in order for them to conclude on their effectiveness.

Notwithstanding the identified material weakness in 2014 described above, our management does not believe that these deficiencies had an adverse effect on our reported operating results or financial condition and management has determined that the financial statements and other information included in this Quarterly Report on Form 10-Q and other periodic filings present fairly in all material respects our financial position and results of operations at and for the periods presented in accordance with GAAP.

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Changes in Internal Control over Financial Reporting

As of March 31, 2015, we have implemented procedures designed to address the material weakness disclosed in our Annual Report on Form 10-K for the year ended December 31, 2014 related to the design and operating effectiveness of certain management review controls. During the three-month period ended March 31, 2015, management has implemented additional and more precise general and management review controls to ensure that all available information is properly considered and reconciled as part of our plan to remediate our material weakness in the year ending December 31, 2015. As we continue to evaluate and work to improve our internal control over financial reporting, management may determine to take additional measures to address this material weakness or to modify the remediation steps described above. We will not be able to assess whether the steps we have taken will fully remediate the material weakness in our internal control over financial reporting until sufficient time passes in order to conduct multiple tests and evaluate their effectiveness. We anticipate that our implementation efforts should be completed and sufficient time shall have passed as of our financial reports for December 31, 2015 and plan to update our assessments and conclusions at that time as to the status of the material weakness.

Other than as described above, there have not been any changes in our internal control over financial reporting (as such term is defined in Rule 13a-15(f) under the Exchange Act) during the period covered by this Quarterly Report on Form 10-Q that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

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PART II - OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS.

We are not subject to any material legal proceedings.

ITEM 1A. RISK FACTORS.

An investment in our common stock involves a high degree of risk. Before investing in our common stock, you should carefully consider the following risk factors together with all of the other information included in this Quarterly Report on Form 10-Q as well as our other publicly available filings with the SEC. If any of these risks occur, our business, results of operations and financial condition could be harmed, the price of our common stock could decline, and you may lose part or all of your investment.

Risks Associated with Commercialization and Product Development

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

PROCYSBI is our only product currently approved for marketing, and as a result, our revenue and operating results substantially depend on the continued commercial success of PROCYSBI. We commenced marketing for PROCYSBI in the United States in June 2013 and Europe in April 2014. We did not have prior experience commercializing therapeutics. In the United States, we are permitted to market PROCYSBI only for the management of nephropathic cystinosis in adults and children six years and older. In September 2013, we received marketing authorization from EC to commercialize PROCYSBI for the treatment of proven nephropathic cystinosis in the EEA. However, we only recently commenced commercial sales of PROCYSBI in select countries in Europe, and have no assurance of securing reimbursement and subsequently launching in additional countries in the EEA. We believe that our results of operations and, in particular, net product sales of PROCYSBI will affect the trading price of our common stock substantially. If PROCYSBI sales do not meet expectations, our stock price may fluctuate, including a potential and possibly significant decrease.

The successful commercialization of PROCYSBI will depend on several factors, including:

- our ability to provide acceptable evidence of the safety and efficacy of PROCYSBI;
- compliance with regulatory requirements, including fulfilling post-approval commitments;
- our ability to obtain approval by regulatory agencies in other countries, including appropriate product labeling;
- the effect of current and future healthcare laws;
- the manufacture and supply of adequate quantities of PROCYSBI in compliance with current good manufacturing practices, or GMPs, as needed to meet commercial demand;
- adequate coverage and reimbursement for PROCYSBI from commercial health plans and government health programs, which we refer to collectively as “third-party payors”;
- our ability to obtain acceptable prices in EEA countries and other select territories, including reimbursement at the country-specific price;

- limitations or warnings currently contained in or as may later be required in approved labeling and the breadth of product labeling or product insert requirements;

- our ability to enter into agreements with wholesalers, distributors and pharmacies on commercially reasonable terms; and

- the protection, development and maintenance of intellectual property and other commercial product protection for PROCYSBI.

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

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

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

- the efficacy, safety, availability and ease of administration of PROCYSBI relative to alternative treatments;
- the price of our product, both in absolute terms and relative to alternative treatments;
- the timing of market introduction of our product relative to competitive drugs;
- the nature of publicity related to our products relative to the publicity related to our competitors' products;
- the prevalence and severity of adverse side effects of PROCYSBI;
- continued patient adherence to therapy;
- availability of coverage and adequate reimbursement from third-party payors;
- provision of affordable out-of-pocket costs to patients and/or other programs to ensure patient access to PROCYSBI; and
- the identification of currently diagnosed and undiagnosed patients and the continued growth of the cystinosis market.

Our efforts to educate patients, physicians, parents, the medical community and third-party payors on the benefits of PROCYSBI may require significant resources and may not be successful. If PROCYSBI does not achieve and maintain significant market acceptance among physicians, patients, patient families, third-party payors or the healthcare community, our business, results of operations and financial condition will be materially adversely affected.

The amount of our product sales of PROCYSBI in the EEA is dependent in part upon the pricing and reimbursement guidelines adopted in each of the EEA countries, which may not be at acceptable levels.

We currently sell PROCYSBI in select EEA countries at the German price. One or more EEA countries may not support our anticipated pricing and reimbursement levels for PROCYSBI, particularly in light of the budget crises faced by a number of countries and third-party payors in the EEA, which would negatively affect revenues from PROCYSBI. The pricing and reimbursement process in EEA countries can be lengthy and involved, and we do not have significant experience with this process. Failure to timely complete the pricing and reimbursement process in the EEA countries will delay our ability to further market PROCYSBI and our ability to derive revenues from that region.

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

products.

Even after we achieve regulatory approvals, we are subject to ongoing obligations and continued regulatory review with respect to our manufacturing processes, labeling, packaging, distribution, storage, adverse event reporting, dispensation, advertising, promotion and recordkeeping. These requirements include submissions of safety and other post-marketing information and reports, ongoing maintenance of product registration and continued compliance with good manufacturing practices, or GMPs, good clinical practices, or GCPs, good distribution practices, or GDPs, and good laboratory practices, or GLPs. If we, our products or product candidates, or the manufacturing facilities for our products or product candidates fail to comply with applicable regulatory requirements, a regulatory agency may:

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- impose restrictions on the marketing or manufacturing of a product, suspend or withdraw product approvals, revoke necessary licenses or suspend product reimbursement;

- impose injunctions, suspensions or revocations of necessary approvals or other licenses;

- issue warning letters, show cause notices or untitled letters describing alleged violations, which may be publicly available;

- suspend any ongoing clinical trials;

- delay or refuse to approve pending applications or supplements to approved applications filed by us;

- refuse to permit drugs or precursor chemicals to be imported or exported to or from the United States;

- suspend or impose restrictions on operations, including costly new manufacturing requirements;

- seize or detain products or require us to initiate a product recall; or

- commence criminal investigations and prosecutions.

Moreover, existing regulatory approvals and any future regulatory approvals that we obtain will be subject to limitations on the approved indicated uses for which the product may be marketed, the conditions of approval, requirements for potentially costly, post-market testing and requirements for surveillance to monitor the safety and efficacy of the product. In the EEA, the advertising and promotion of pharmaceuticals is strictly regulated. The direct-to-consumer promotion of prescription pharmaceuticals is not permitted, and some countries of the EEA require the notification and/or prior authorization of promotional or advertising materials directed at healthcare professionals. The FDA, EMA, EC and other authorities in the EEA countries strictly regulate the promotional claims that may be made about prescription products, and our product labeling, advertising and promotion are subject to continuing regulatory review. Physicians nevertheless may prescribe our product to their patients in a manner that is inconsistent with the approved label or that is off-label. Positive clinical trial results in any of our RP103 programs increase the risk that immediate-release cysteamine bitartrate may be used off-label in those indications in certain geographic areas due to the lower cost of immediate-release cysteamine bitartrate. If we are found to have improperly promoted off-label uses, we may be subject to significant sanctions, civil and criminal fines and injunctions prohibiting us from engaging in specified promotional conduct.

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

The regulations, policies or guidance of regulatory agencies may change and new or additional statutes or government regulations may be enacted that could prevent or delay regulatory approval of our product candidates or further restrict or regulate post-approval activities. For example, the Food and Drug Administration Safety and Innovation Act (“FDASIA”), requires the FDA to issue new guidance describing its policy regarding internet and social media promotion of regulated medical products, and the FDA may soon specify new restrictions on this type of promotion. In January 2014, the FDA released draft guidance on how drug companies can fulfill their regulatory requirements for

post-marketing submission of interactive promotional media, and though the guidance provided insight into how the FDA views a company's responsibility for certain types of social media promotion, there remains a substantial amount of uncertainty. We cannot predict the likelihood, nature or extent of adverse government regulation that may arise from pending or future legislation or administrative action, either in the United States or abroad. If we are unable to achieve and maintain regulatory compliance, we may not be permitted to market our drugs, which would adversely affect our ability to generate revenue and achieve or maintain profitability.

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

The research, testing, manufacturing, labeling, approval, sale, marketing and distribution of drug products, among other things, are subject to extensive regulation by the FDA and other regulatory authorities in the United States and by similar foreign regulatory governmental entities. We are not permitted to market any of our drug product candidates unless we obtain and maintain appropriate pre-market approvals from regulatory agencies in each of the markets in which we intend to market our products. Once approved, we may only market our products for the specific uses that are reflected in the product's approved labeling. A product's approved labeling may contain limitations or warnings or may be for different patient populations or for fewer or more limited indications than what we requested in our pre-market approval application, which could result in reimbursement complications, limit access for intended use or limit the commercial profile of the drug. In the United States, we are permitted to market the active pharmaceutical ingredient of RP103 in the formulation of a final drug product and in the doses approved under the brand name PROCYSBI only for the management of nephropathic cystinosis in adults and children six years and older under the brand name PROCYSBI. We are permitted to market PROCYSBI in the EEA as an orphan medicinal product for the treatment of proven nephropathic cystinosis. We do not have approval of RP103 in any other market nor for any other disease indication. There can be no assurance that we will obtain regulatory approval for any other uses for PROCYSBI or any of our other product candidates or that, even if we obtained additional approvals, we would be able to commercialize the product candidates successfully.

A new drug application ("NDA"), submitted to the FDA, or a marketing authorization application ("MAA"), submitted to the EMA, must be supported by extensive clinical and preclinical data, as well as extensive information regarding chemistry, manufacturing and controls. This information must demonstrate the safety and efficacy of the applicable product candidate for the management of each individual indication to the satisfaction of the applicable regulatory authority. Obtaining approval of an NDA, MAA or any other filing for marketing authorization in a foreign country is an extensive, expensive and uncertain process. The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. The FDA, EC or other regulatory authorities may delay, limit or deny approval of RP103 or our future drug product candidates for many reasons, including:

- the results of clinical trials may not meet the level of statistical or clinical significance required by regulatory authorities for approval;

- regulatory authorities may disagree with the number, design, size, conduct or implementation of our clinical trials; they may change the requirements for approval even after having reviewed and commented on the design for our clinical trials;

- regulatory authorities may not find the data from preclinical studies and clinical trials sufficient to demonstrate that product candidates have adequate clinical and other benefits or adequate safety profiles, even if they achieve their specified endpoints in clinical trials; or they may disagree with our interpretation of data from preclinical studies or clinical trials and require that we conduct additional trials;

- regulatory authorities may not accept data generated at our clinical trial sites;

- regulatory authorities may have difficulties scheduling an advisory committee meeting (or equivalent, if required) in a timely manner, or the advisory committee may recommend against approval of our application or may recommend that the regulatory agency require, as a condition of approval, additional preclinical studies or clinical trials; approval may also be contingent on a Risk Evaluation and Mitigation Strategy, which limits the labeling, distribution or

promotion of a drug product;

- regulatory authorities may require additional preclinical or clinical studies or other data prior to granting approval, and we may not be able to generate the required data on a timely basis, if at all;

- regulatory authorities may identify deficiencies in the manufacturing processes or in the facilities of our third-party suppliers and/or contract manufacturers or may require us to manufacture additional validation batches or change our process, specifications or third-party suppliers or contract manufacturers; and

- we may not be able to validate our manufacturing process to the satisfaction of the regulatory authorities, or they may not agree with our plan for potential retrospective validation.

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

We do not have internal manufacturing capabilities. Throughout 2015, we expect to continue to rely on a single source supplier for our active pharmaceutical ingredient ("API"), and throughout most of 2015, a single third-party manufacturer for the conversion to finished commercial drug product. We also rely on third parties for the distribution and pharmaceutical services of PROCYSBI in the United States and the EEA. If we are unable to rely on these third parties, our revenue will be delayed or diminished and our business, results of operations and financial condition will be materially adversely affected.

We do not own or operate manufacturing facilities and currently lack the in-house capability to manufacture PROCYSBI or RP103. As a result, we currently contract with external contract manufacturing organizations ("CMOs"), for commercial and clinical quantities of PROCYSBI and RP103 for the indications under development. We rely on a single source supplier for our cysteamine API. While we have procured additional manufacturing support with a second supplier for clinical supply of our finished drug product, for the majority of 2015, we will continue to rely on a single third-party manufacturer for supply of finished commercial products until the second supplier can be validated and provide finished product. Our ability to obtain sufficient quantities of PROCYSBI and RP103 is constrained by limited supplies of raw materials and the limited capacity and output of these third parties. Furthermore, any reduction, delay or interruption in our supply of API from the single source supplier or of our supply of finished goods from our CMO, together with any additional required efforts to identify and qualify alternative sources of API supply, could result in significant additional operating costs, interruptions in product supply, delays in sales of PROCYSBI and delays in developing RP103 for additional indications. In addition, supply arrangements from alternative sources not currently under contract may not be available on acceptable economic terms, if at all.

The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production to commercial requirements. Difficulties may arise related to internal processes, production costs and yields, quality control, including stability of the product and quality control testing, sourcing scarcities, resource constraints, equipment problems, shortages of qualified personnel, labor disputes, severe weather events, unstable political environments or financial difficulties at foreign facilities, as well as compliance with strictly enforced federal, state and foreign regulations. Manufacturers may breach their agreements with us or may terminate or decline to renew their agreements with us, whether due to our breach of the relevant agreements or based on their own business priorities. In addition, due to our small patient population, the manufacture of our drug may be given lower prioritization on the production line if manufacturing prioritization is decided by scale.

Manufacturers and suppliers are subject to regulatory requirements covering, among other things, manufacturing, process controls, testing, quality control and record keeping and are subject to ongoing inspections by regulatory agencies. We have no direct control over the ability of our contract manufacturing parties to maintain adequate quality control, quality assurance and qualified personnel, and while final outputs are reviewed by our own internal quality control, we depend on our third-party supplier and manufacturers for compliance with the FDA's current cGMP requirements and other FDA requirements, the Drug Enforcement Administration's regulations and other rules and regulations prescribed by applicable non-U.S. regulatory authorities. If our contract manufacturing partners cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or other regulatory authorities, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities, and we may experience long delays and interruptions to our manufacturing supply and increased costs.

Pursuant to ongoing obligations from our NDA for PROCYSBI, we are required to collect and submit data to the FDA regularly regarding our currently observed clinical and commercial product profile and overall product safety assessment. In addition, we intend to continue to evaluate our product specification limits, and any changes to our product specifications may require additional review and approval by regulators in the United States and Europe. If there are material delays in any such review and approval process, or if regulators reject any proposals for changes in product specifications or require additional data to support the updated specifications, we may experience an inventory shortfall, which would have a material adverse effect on sales of PROCYSBI.

If we or our third-party suppliers and manufacturers fail to comply with applicable regulatory requirements, we could experience significant delays or interruptions to our manufacturing supply that may result in the delay or suspension of our preclinical or clinical trials. In addition, a regulatory agency could issue warning letters or untitled letters, seek an injunction, impose civil or criminal penalties or monetary fines, suspend or withdraw regulatory approval, suspend any ongoing clinical trials, refuse to approve pending applications or supplements to applications, suspend or impose restrictions on operations, including costly new manufacturing requirements, seize or detain products or request that we initiate a product recall.

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

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

If serious adverse side effects become associated with PROCYSBI, our business, results of operations and financial condition will be materially adversely affected.

The prescribing information for PROCYSBI includes several warnings relating to observed adverse reactions of cysteamine bitartrate usage. The FDA may require products approved under Section 505(b)(2) of the Federal Food, Drug and Cosmetic Act (the "FDCA"), to bear the same or similar warning statements as the reference product used in the approval. We expect to update adverse reactions listed in the prescribing information based on continued commercial use and additional clinical trials. If additional adverse reactions emerge, or if there is a pattern of severe or persistent previously observed side effects in the relevant patient populations, the FDA, the European Medicines Agency (the "EMA") or other regulatory agencies could modify or revoke our marketing approval, require us to modify our label or require us to suspend production, require a product recall, or we may choose to withdraw PROCYSBI from the market. Regulatory authorities could also require us to change the way the product is administered or modify the product in some other way, or they could require us to conduct additional clinical trials or costly post-marketing testing and surveillance to monitor the safety or efficacy of the product. If this were to occur, we may be unable to maintain marketing approval in our approved indications and/or obtain marketing approval in other indications. In addition, patients or their representatives may bring claims against us alleging serious adverse side effects or harm suffered as a result of use of PROCYSBI. Any such side effects or related claims could have a material adverse effect on our business, results of operations and financial condition. See also the risk factor titled "We may be subject to product liability claims."

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

Clinical trials are very expensive, time consuming and difficult to design and implement. The outcome of clinical trials is uncertain, and results of earlier studies and trials may not be predictive of future trial results. Delays in the commencement or completion of clinical testing for RP103 or preclinical or clinical testing for any of our other product candidates could significantly affect our product development costs and business plan.

Preclinical studies involve testing in appropriate multiple non-human disease models to demonstrate efficacy and safety. Regulatory agencies evaluate these data carefully as part of their determination whether to authorize clinical testing in humans. If certain preclinical data reveal potential safety issues or if the results are inconsistent with an expectation of the drug product candidate's efficacy in humans, the regulatory agencies may require additional testing before allowing human clinical trials. This additional testing will increase program expenses and extend timelines. There are many potential preclinical models to test for different disease states, and we could fail to choose the best

preclinical model to determine proof of concept, safety and efficacy of our drug product candidates. 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.

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Following successful preclinical testing, drug product candidates must be tested in a clinical development program to provide data on safety and efficacy in humans prior to becoming eligible for product approval and licensure by regulatory agencies. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. Clinical trials may also be delayed or repeated as a result of ambiguous or negative interim results or unforeseen complications in testing. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. The clinical trial process may fail to demonstrate with statistical significance that our drug product candidates are safe for humans and effective for indicated uses. This failure may cause us to abandon a drug product candidate and may delay development of other drug product candidates. Any delay in, or termination of, our preclinical testing or clinical trials will delay the filing of relevant marketing applications with regulatory agencies and, ultimately, our ability to commercialize our drug product candidates and generate revenues from related products.

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 nature of the disease or medical condition being studied, the availability of alternative therapies and drugs, the proximity of patients to clinical sites and the eligibility criteria for the study. Delays in planned patient enrollment may result in increased costs and delays. In addition, because many of our clinical trials involve small patient populations, the results of these early clinical trials may not be indicative of future results.

Under the Prescription Drug User Fee Act, the FDA seeks to respond to NDAs within ten months of the filing date, but this timeframe is often extended. For example, a sponsor may seek FDA designation of a drug candidate as a “fast track product.” Fast track products are those products intended for the treatment of a serious or life-threatening disease or condition and which demonstrate the potential to address unmet medical needs for such disease or condition. If fast track designation is obtained, the FDA may initiate review of sections of an NDA before the application is complete. This “rolling review” is available if the applicant provides and the FDA approves a schedule for the remaining information. In addition, FDASIA established a new category of drugs referred to as “breakthrough therapies,” which are defined as drugs intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. In the future, we may request breakthrough designation or fast track designation from the FDA for our other drug product candidates, but there can be no assurance that we will obtain such designations. Moreover, even if we obtain breakthrough designation or fast track designation from the FDA, the designations do not guarantee that the FDA will approve our NDA, that the development program or review timeline will ultimately be shorter than if we had not obtained the designations or that the FDA will not request additional information, including additional clinical studies, during its review.

We do not know whether our investigational new drug, or IND, applications 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 and completion 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;

- inability to design appropriate clinical trial protocols;

- delays or failures in reaching agreement on acceptable clinical trial agreement terms or clinical trial protocols with prospective sites;

- inability of our clinical research organizations ("CROs"), or other third-party contractors to comply with all contractual requirements or to perform their services in a timely or acceptable manner;

- inability by us, our CROs or their employees to conduct the clinical trial in accordance with all applicable FDA, Drug Enforcement Administration or other regulatory requirements or our clinical protocols;

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- lack of efficacy during, or other unfavorable results from, clinical trials or preclinical studies;
- discovery of serious or unexpected toxicities or side effects experienced by study participants or other unforeseen safety issues;
- failure of patients to complete the clinical trial, or inability or unwillingness of patients or medical investigators to follow our clinical trial protocols;
- inability to monitor patients adequately during or after treatment;
- regulatory action by the FDA or other regulatory authorities; and
- lack of adequate funding to continue the clinical trial, including the incurrence of any unforeseen costs.

In addition, changes in applicable regulatory requirements and guidance may occur, and we may need to amend clinical trial protocols to reflect these changes. Amendments may require us to resubmit our clinical trial protocols to Institutional Review Boards for reexamination, which may affect the costs, timing or successful completion of a clinical trial.

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

Any delay in our preclinical or clinical programs or the failure to demonstrate safety or efficacy in our clinical trials would have a material adverse effect on our business, results of operations and financial condition.

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

PROCYSBI has received marketing approval from the FDA for the management of nephropathic cystinosis in adults and children six years and older and seven years of market exclusivity as an orphan drug in the United States. PROCYSBI has also received approval as an orphan medicinal product for the management of proven nephropathic cystinosis and 10 years of market exclusivity in the EEA. As part of our business strategy, we intend to develop RP103, and potentially other drugs, for additional therapeutic indications that may be eligible for FDA and EMA orphan drug designation.

Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, defined as a patient population of less than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated condition for a period of seven years, plus an additional six months if designated for a pediatric indication.

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In the EU, the EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the EU and for which no satisfactory method of diagnosis, prevention or treatment has been authorized (or if the product would be a significant benefit to those affected). In addition, designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition as well as when it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the medicinal product without incentives. An EU orphan drug designation entitles a party to financial incentives such as reduced fees or fee waivers, and 10 years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. An applicant must request orphan drug designation before submitting an application for marketing approval.

Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Even though we have been granted orphan drug designation in the United States and in the EU prior to the approval of RP103 for the potential treatment of HD, and even if we obtain orphan drug designation for our future drug product candidates, we may not fulfill the criteria for exclusivity, or we may not be the first to obtain marketing approval for any orphan indication. Further, even if we obtain orphan drug exclusivity for a particular product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve a drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. Orphan drug exclusive marketing rights may also be lost if the FDA later determines that the request for designation was materially defective, if a subsequent product is deemed clinically superior or if the manufacturer is unable to deliver sufficient quantities of the drug.

Because the extent and scope of patent protection for some of our drug products may be particularly limited, orphan drug designation is especially important for our eligible products and we plan to rely on the orphan exclusivity period to maintain a competitive position. However, if we do not obtain and/or maintain orphan drug exclusivity for RP103, or if our drug products do not have strong patent protection, our competitors may sell the same drug to treat the same condition, possibly at lower prices, and our revenues will be reduced. Also, without strong patent protection, competitors may sell a generic version of our products upon the expiration of orphan exclusivity if our patent position is not upheld.

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 technologies similar to those that we are pursuing and are developing pharmaceutical products that are competitive with PROCYSBI or our drug product candidates. Many of the pharmaceutical companies in areas competitive with us have greater capital resources, larger overall research and development staff and facilities and a longer history in drug discovery, development, regulatory approval, manufacturing and marketing than we do. With these additional resources and experience, our competitors may be able to respond to rapid and significant technological changes in the biotechnology and pharmaceutical industries faster than we can.

We expect that the technologies associated with biotechnology research and development will continue to develop rapidly. Our future success will depend in large part on our ability to maintain a competitive position with respect to these technologies. Rapid technological development, as well as new scientific developments, may result in our compounds, drug products, drug product candidates or processes becoming obsolete before we can recover any or all of the expenses incurred to develop them. For example, changes in our understanding of the appropriate population of

patients who should be treated with a targeted therapy like those 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.

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

PROCYSBI and our clinical development of RP103 target diseases with small patient populations, including cystinosis and HD respectively. To successfully commercialize a drug product for these indications, we must identify patients and a targeted prescriber base for the drug product. Due to small patient populations, we believe that we would need to have significant market penetration to achieve meaningful revenues. In addition, the per-patient prices at which we sell PROCYSBI and RP103 for these indications will need to be relatively high in order for us to generate an appropriate return for the investment in these product development programs and to achieve meaningful gross margins. Patients who discontinue therapy or do not fill prescriptions are not easily replaced by new patients because of the limited patient population. There can be no assurance that we will successfully obtain or maintain sufficient market share or per-patient prices. Because the potential target populations are very small, even if we obtain significant market share for PROCYSBI and RP103, we may never achieve profitability despite obtaining such significant market share.

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Our development strategy for RP103 depends upon the FDA's prior findings of safety and effectiveness of cysteamine bitartrate based on data not developed by us but upon which the FDA may rely in reviewing any future NDA.

The Hatch-Waxman Amendments added to the FDCA Section 505(b)(2), which permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. Under this statutory provision, the FDA may rely, for purposes of approving an NDA, on safety and effectiveness data not developed by the filer of the NDA. We also plan to submit an NDA for RP103 for approval of other indications under Section 505(b)(2), and if we are able to submit those NDAs, they will rely, in part, on the FDA's previous findings of safety and effectiveness for cysteamine bitartrate. Even though we may be able to take advantage of Section 505(b)(2) to support potential U.S. approval for these additional product candidates, the FDA may require us to perform additional studies or measurements to support approval. In addition, the FDA's interpretation and use of Section 505(b)(2) has been controversial and has previously been challenged in court, though without a definitive ruling on the propriety of the FDA's approach. Future challenges, including a direct challenge to the approval of our products and product candidates, may be possible and, if successful, could limit or eliminate our ability to rely on the Section 505(b)(2) pathway for the approval of RP103 and our other product candidates. Such a result could require us to conduct additional testing and costly clinical trials, which could substantially delay or prevent the approval and launch of any future product candidates and would materially adversely affect our business, results of operation and financial condition.

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

We participate in the Medicaid Drug Rebate Program, as administered by the Centers for Medicare & Medicaid Services ("CMS"), and other federal and state government pricing programs in the United States, and we may participate in additional government pricing programs in the future. These programs generally require us to pay rebates or otherwise provide discounts to government payors in connection with drugs, including PROCYSBI, that are dispensed to beneficiaries of these programs. In some cases, such as with the Medicaid Drug Rebate Program, the rebates are based on pricing and rebate calculations that we report on a monthly and quarterly basis to the government agencies that administer the programs. Pricing and rebate calculations are complex, vary among products and programs, and are often subject to interpretation by governmental or regulatory agencies and the courts. The terms, scope and complexity of these government pricing programs change frequently. Responding to current and future changes may increase our costs and the complexity of compliance will be time consuming. See also the risk factor titled "Legislative changes regarding manufacturers' rebate obligations for new formulations of oral solid dosage form drugs under the Medicaid Drug Rebate Program, if applied to PROCYSBI, would have a material adverse effect on our business, results of operations and financial condition."

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

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

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Pressure from third-party payor coverage, reimbursement and pricing policies may impair our customers' ability to be reimbursed for PROCYSBI and our other future product candidates at adequate prices or on adequate terms, which may in turn materially adversely affect our business, results of operations and financial condition.

Market acceptance and sales of PROCYSBI and any product candidates that we may develop will depend in large part on third-party payor coverage and reimbursement policies and may be affected by future healthcare reform measures in the United States, the EEA countries and other key international markets. The continuing efforts of governmental and other third-party payors to contain, reduce or shift the costs of healthcare through various means, including an increased emphasis on managed care and attempts to limit or regulate the price of medicinal products and services, particularly for new and innovative products and therapies, may result in downward pressure on pricing, reimbursement and utilization, which may adversely affect our product sales and results of operations. Moreover, because private health insurers and other third-party payors often follow the coverage and reimbursement policies of government payors, including the Medicare and Medicaid programs, cost-containment measures under these programs play a particularly significant role in the reimbursement landscape. The government programs relevant to our products include, without limitation, the following:

- the Medicaid Drug Rebate Program, under which manufacturers must report pricing information and pay rebates in order for their drug products to be covered under state Medicaid programs;

- the Public Health Service's 340B Drug Pricing Program, under which manufacturers must offer discounts to certain healthcare organizations that care for underserved populations;

- the Department of Veterans Affairs' Federal Supply Schedule pricing program, under which manufacturers agree to offer drugs to certain governmental providers at reduced rates;

- the TRICARE Retail Pharmacy Program, under which manufacturers must agree to honor certain discounted prices, specifically Federal Ceiling Prices under the Veterans Health Care Act, as a condition for placement in the Department of Defense uniform formulary; and

- the Medicare Part D program, under which manufacturers contract with plan sponsors to offer certain outpatient drugs to Medicare beneficiaries.

In addition, in the United States, third-party payors often develop cost containment measures using policies that specifically target specialty products and high-cost drugs. For example, formulary placements may be less favorable for brand and higher-costing drugs, which may result in, among other things, greater out-of-pocket costs to patients. PROCYSBI often is subject to such measures, and similar future policies addressing such cost-containment measures may also affect PROCYSBI.

Further, third-party payors also are increasingly considering new metrics as the basis for reimbursement rates, such as average sales price, AMP or actual acquisition cost, and for cost-benefit analyses with comparable drugs. Although the changes to reimbursement methodologies are generally intended to limit payment increases, it is difficult to project the impact of these and other alternative reimbursement methodologies on the willingness of payors to reimburse PROCYSBI and any product candidates that we may develop. To date, PROCYSBI generally has been covered and reimbursed in the United States and the select countries we have entered in Europe, but we do not know whether third-party payors will continue to cover and reimburse PROCYSBI in these markets or at the level PROCYSBI is currently covered, will reimburse PROCYSBI in other EEA countries or will reimburse RP103 and our future products until we enter into payor negotiations. If coverage and reimbursement are not available or limited, or reimbursement is available only at limited levels, our business, results of operations and financial condition will be materially adversely affected.

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

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

In 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, which we refer to together as the "Affordable Care Act," was adopted. This law intends to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The Affordable Care Act, among other things:

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- increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program;

- revised the definition of AMP for reporting purposes, which could further increase the amount of rebates paid by manufacturers under the Medicaid Drug Rebate Program;

- extended the Medicaid Drug Rebate Program to beneficiaries enrolled in Medicaid managed care organizations;

- addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected and for oral solid line extensions and reformulated drugs, which, depending on how the provision is interpreted and implemented, could increase our Medicaid rebate rate substantially;

- imposed a significant annual fee on companies that manufacture or import branded prescription drug products and established an annual non-deductible fee on entities that sell branded prescription drugs or biologics to specified government programs in the United States;

- expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance; and

- included a 50% point-of-sale discount off negotiated prices on applicable brand-name drugs for Medicare Part D participants in the coverage gap, or "donut hole," as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

Other legislative and regulatory changes have also been proposed and adopted in the United States since the enactment of the Affordable Care Act. On August 2, 2011, the Budget Control Act of 2011 created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These automatic reductions included aggregate reductions of Medicare payments to providers of 2%. These reductions went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2024 unless additional Congressional action is taken.

In addition, legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. Increased scrutiny by the U.S. Congress of the FDA's approval process may subject us to more stringent product labeling and post-marketing testing and other requirements, and delays in feedback from the FDA may affect our ability to update or adjust our label in a timely manner in the interest of patient adherence and tolerability. We cannot predict whether other legislative changes will be adopted or how such changes would affect the pharmaceutical industry generally and the commercialization of PROCYSBI specifically.

Legislative changes regarding manufacturers' rebate obligations for new formulations of oral solid dosage form drugs under the Medicaid Drug Rebate Program, if applied to PROCYSBI, would have a material adverse effect on our business, results of operations and financial condition.

The Affordable Care Act created a new formula to determine the rebate amount owed by manufacturers of "line extension" drugs that would likely lead to higher rebates owed by such manufacturers under the Medicaid Drug Rebate Program. The Affordable Care Act defined a line extension drug to mean a new formulation of a drug, "such as an extended release formulation." In April 2010, CMS stated that it would issue additional guidance to manufacturers and other stakeholders concerning line extensions of existing drugs. In 2012, in implementing the new law, CMS proposed a broad definition of a line extension drug to include any single source or innovator multiple source drug that is an

oral solid dosage form approved by the FDA as a change to the initial brand name listed drug; a new formulation of a previously approved oral solid dosage form drug; a new combination of two or more oral solid dosage form drugs; or a new indication for an already marketed oral solid dosage form drug. In the proposed rule, orphan drugs were included as part of the definition of a line extension drug. Although CMS has not yet issued a final rule, in the event that CMS finalizes the rule as currently proposed, PROCYSBI would likely be subject to the new rebate calculations under the Medicaid Drug Rebate Program, and, as a result, PROCYSBI sales to Medicaid beneficiaries could be reimbursed at cost and any profits from those sales could be eliminated. Approximately 20% of our current PROCYSBI sales are to Medicaid beneficiaries. Accordingly, the implementation of the proposed rules may have a material adverse effect on our business, results of operations and financial condition.

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We are subject, directly and indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, physician payment transparency laws, corrupt practices and bribery laws and health information privacy and security laws. Failure to comply with these laws may subject us to substantial penalties.

We do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors. However, federal and state healthcare laws and regulations pertaining to fraud and abuse, physician payment transparency and privacy and security laws and regulations apply to us and our arrangements with healthcare providers, customers and other entities, including our marketing practices, educational programs and pricing policies. These laws include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in exchange for or to induce either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs, such as the Medicare and Medicaid programs. A person or entity does not need to have actual knowledge of the federal Anti-Kickback Statute or specific intent to violate it to have committed a violation; in addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;

- federal false claims laws, including, without limitation, the False Claims Act, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid or other federal third-party payors that are false or fraudulent, such as engaging in improper promotion of products for off-label uses;

- the federal Civil Monetary Penalties law, which prohibits, among other things, offering or transferring remuneration to a federal healthcare beneficiary that a person knows or should know is likely to influence the beneficiary's decision to order or receive items or services reimbursable by the government from a particular provider or supplier;

- federal criminal laws that prohibit executing a scheme to defraud any federal healthcare benefit program or making false statements relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it to have committed a violation;

- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information;

- the federal Physician Payment Sunshine Act, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS information related to payments or other "transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, and requires applicable manufacturers and group purchasing organizations to report annually to CMS ownership and investment interests held by the physicians described above and their immediate family members and payments or other "transfers of value" to such physician owners (manufacturers are required to submit reports to CMS by the 90th day of each calendar year);

- in the EEA, in various member states including France, the United Kingdom, the Netherlands, Italy and Spain, rules adopted by the legislator or self-regulatory industry bodies requiring the notification and/or publication of certain transfers of value from pharmaceutical companies to healthcare professionals (for example, France has recently adopted legislation (Law No. 2011-2012, or the "French Sunshine Act," and Decree no. 2013-414 which implements it) requiring pharmaceutical companies to disclose and publish agreements with or transfers of value to healthcare

professionals);

- the U.S. Foreign Corrupt Practices Act (the "FCPA"), which prohibits corporations and individuals from paying, offering to pay or authorizing the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity; the UK Bribery Act, which prohibits both domestic and international bribery, as well as bribery across both public and private sectors; and bribery provisions contained in the German Criminal Code, which, pursuant to draft legislation being prepared by the German government, may make the corruption and corruptibility of physicians in private practice and other healthcare professionals a criminal offense; and

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- analogous state and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. In addition, as we expand our development and commercialization activities outside of the United States, we will need to establish and expand business relationships with various third parties, such as independent contractors, distributors, vendors, advocacy groups and physicians, and we will interact more frequently with foreign officials, including regulatory authorities and physicians employed by state-run healthcare institutions who may be deemed to be foreign officials under the FCPA, the UK Bribery Act or similar laws of other countries that may govern our activities. Any interactions with any such parties or individuals where compensation is provided that are found to be in violation of such laws could result in significant civil and criminal penalties.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available under such laws, it is possible that some of our business activities, including our relationships with physicians and other healthcare providers, some of whom recommend, purchase and/or prescribe our products, and the manner in which we promote our products, could be subject to challenge under one or more of such laws. We are also exposed to the risk that our employees, independent contractors, principal investigators, consultants, vendors, distributors and CROs may engage in fraudulent or other illegal activity. While we have policies and procedures in place prohibiting such activity, misconduct by these parties could include, among other infractions or violations, intentional, reckless and/or negligent conduct or unauthorized activity that violates FDA regulations, including those laws that require the reporting of true, complete and accurate information to the FDA, manufacturing standards, federal and state healthcare fraud and abuse laws and regulations, laws that require the true, complete and accurate reporting of financial information or data or other commercial or regulatory laws or requirements. It is not always possible to identify and deter misconduct by our employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If our operations are found to violate any of the laws described above or any other laws and regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, disgorgement, the curtailment or restructuring of our operations, the exclusion from participation in federal and state healthcare programs and imprisonment of officers involved, any of which could adversely affect our ability to market PROCYSBI, RP103 and other future drug candidates, once approved, and materially adversely affect our business, results of operations and financial condition. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. See also the risk factor titled "If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the United States, we could be subject to additional reimbursement requirements, penalties, sanctions and fines which could have a material adverse effect on our business, results of operations and financial condition."

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

In the course of product development, we engage and collaborate with a variety of external organizations to perform services essential to drug product development. The organizations which perform services may include, but are not limited to: governmental agencies and university laboratories; other biotechnology and pharmaceutical companies; CMOs; CROs; distribution and supply (logistics) service organizations; contract testing organizations; consultants or consulting organizations with specialized knowledge based expertise; and intellectual property law firms.

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

Specifically, we have and will continue to rely on third parties, such as CROs and/or co-operative groups, to assist us in overseeing and monitoring clinical trials as well as to process the clinical results. Any failure of such third parties to perform or to meet the applicable standards will result in delays in or failures to complete trials. A failure by such third parties to observe the terms of a product development program for any particular product candidate or to complete the clinical trials for a product candidate in the anticipated time frame could materially adversely affect our business, results of operations and financial condition.

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

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

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

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

We must establish and maintain relationships with key opinion leaders, leading scientists and research institutions. We believe that such relationships are critical to establishing products as a standard of care for their approved indications. Although we have various medical and scientific advisors and research collaborations, there is no assurance that our

advisors and our research collaborators will continue to work with us or that we will be able to attract additional advisors or collaborators. If we are not able to maintain existing or establish new clinical and scientific relationships to assist in our commercialization and research and development, we may not be able to establish our products as the standard of care or successfully develop our drug product candidates.

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If we fail to maintain an effective system of internal controls, we may not be able to accurately report our financial results and prevent fraud.

The Sarbanes-Oxley Act of 2002 (the "Sarbanes-Oxley Act") requires, among other things, that we maintain effective internal control over financial reporting and disclosure controls and procedures. In particular, we must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act. During its evaluation of the effectiveness of internal control over financial reporting as of December 31, 2014, management identified a material weakness related to our inventory costing and overhead allocations for our commercial product PROCYSBI and determined that our review of our inventory costing and overhead allocations were not performed at a sufficiently detailed level to detect errors in our inventory and related accounts. With the oversight of management and our Audit Committee, we have initiated actions to address the root causes of the material weakness identified in 2014. There can be no assurance that such actions will be sufficient to remedy the material weakness identified or that additional material weaknesses or other control or significant deficiencies will not be identified in the future. If we continue to experience a material weakness in our internal controls or fail to maintain or implement required new or improved controls, such circumstances could cause us to fail to meet our periodic reporting obligations or result in material misstatements in our financial statements, or adversely affect the results of periodic management evaluations and annual auditor attestation reports. Each of the foregoing results could cause stockholders to lose confidence in our reported financial information and lead to a decline in our stock price. See also the section entitled "Controls and Procedures —Changes in Internal Control over Financial Reporting."

We may be subject to product liability claims.

The nature of our business exposes us to potential liability risks inherent in the testing (including through human trials), manufacturing and marketing of drugs. PROCYSBI and our drug product candidates could potentially harm people, and we may be subject to costly and damaging product liability claims regardless of actual harm. Many of the participants in our clinical trials and cystinosis patients who use PROCYSBI are already critically ill or suffering from chronic debilitating diseases. The waivers we obtain from participants in clinical trials may not be enforceable and may not protect us from liability or the costs of product liability litigation.

We may not be able to avoid significant liability if any product liability claim is brought against us. Although we currently carry product liability insurance, it may not be sufficient to cover any claims. We may be unable to maintain product liability insurance in the future at satisfactory rates or in adequate amounts. Regardless of the merits or eventual outcome, product liability claims may result in decreased demand for our product candidates, injury to our reputation, withdrawal of clinical trial participants and inability to continue clinical trials, costs to defend the related litigation, diversion of management's time, substantial monetary awards to trial participants, or patients, regulatory investigations, product recalls or withdrawals, labeling, marketing or promotional restrictions and loss of revenue, any of which may materially adversely affect our business, results or operations and financial condition.

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

Our business strategy, including continued commercial sales of PROCYSBI in the United States and certain countries in the EEA, expansion of our commercial operations into other markets, the continuation of our clinical-stage programs and the in-license and acquisition of additional clinical-stage product candidates, will require us to retain existing and add required new qualified and experienced personnel in multiple functional areas over the next several years.

Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to manage the expansion of our operations effectively, which may result in weaknesses in our infrastructure, operational mistakes,

loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, including additional product candidates.

In addition, in connection with the commercial launch of PROCYSBI in the EEA, we have expanded our operations in Europe where we expect to continue to add personnel. We may encounter difficulties successfully managing remotely a substantially larger and internationally diverse organization and may encounter delays in commercialization if we are not successful in integrating our international operations. Challenges related to managing international operations may arise from staffing and managing foreign operations, reduced or varied protection for intellectual property rights in some countries, potential strain on our financial and managerial controls and reporting systems and procedures, diverse individual country regulatory and statutory laws, the costs of maintaining EEA presence, in-country legal entities and related tax structures, fluctuations in currency exchanges and political and economic instability, including wars, terrorism and political unrest, boycotts, curtailment of trade and other business restrictions.

If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate revenue could be reduced, and we may not be able to implement our business strategy.

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Credit risks from customers outside the United States may materially adversely affect our business, results of operations and financial condition.

Sales of our products to government supported customers outside of the United States are likely to be subject to significant payment delays due to government funding and reimbursement practices, which will result in an increase in the length of time that we may have accounts receivable outstanding. In addition, many governments in Europe are facing significant liquidity crises. If government reimbursement for sales of PROCYSBI or any future products in EEA countries is delayed or becomes unavailable, we may not be able to collect on amounts payable to us in reasonable time frames from such customers, which would cause our capital requirements to increase and would materially adversely affect our business, results of operations and financial condition.

Macroeconomic conditions could materially adversely affect our business, results of operations and financial condition.

Various macroeconomic factors, such as changes in inflation, interest rates, foreign currency exchange rates and overall business and economic conditions and uncertainties, including those resulting from conditions in the global financial markets, could adversely affect our business, results of operations and financial condition. For example, if inflation or other factors were to significantly increase our business costs, it may not be feasible to increase the price of PROCYSBI or any future products due to reimbursement procedures and other pricing pressures.

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

Our product sales in the United States could be reduced by imports from countries where our products are available at lower prices.

Our recognized product sales in the United States may be reduced if PROCYSBI is 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 revenues could be reduced, and our business, results of operations and financial condition could be materially adversely affected.

Our international sales and operating expenses are subject to fluctuations in currency exchange rates.

A portion of our business is conducted in currencies other than our reporting currency, the U.S. dollar. We recognize foreign currency gains or losses arising from our operations in the period in which we incur those gains or losses. As a result, currency fluctuations between the U.S. dollar and the currencies in which we do business cause foreign currency translation gains and losses. Because of the number of currencies that may be involved as we enter new markets, the variability of currency exposures and the potential volatility of currency exchange rates, we may suffer significant foreign currency translation and transaction losses due to the effect of exchange rate fluctuations. We have not entered into derivative instruments to offset the impact of foreign exchange fluctuations. Given the volatility of exchange rates, there is no assurance that we will be able to effectively manage currency transaction and/or conversion risks.

We may engage in strategic transactions that could affect our liquidity, increase our expenses and present significant challenges in focus and energy to our management.

From time to time, we may consider strategic transactions, such as acquisitions of companies, asset purchases and out-licensing or in-licensing of intellectual property, products or technologies. Additional potential transactions that we may consider include a variety of business arrangements, including spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. These transactions may entail numerous operational and financial risks, including exposure to unknown liabilities; disruption of our business and diversion of our management's time and attention in order to develop acquired products or technologies or to conduct business in new markets; use of existing cash reserves, dilutive issuances of equity securities to replenish cash requirements or to directly pay for transactions, or incurrence of substantial debt to pay for acquisitions; higher-than-expected acquisition and integration costs; increases in near- and long-term expenditures; unexpected difficulties or shortcomings in the development or commercialization of acquired assets, products, or businesses; write-downs of assets or goodwill or impairment charges; increased amortization expenses; difficulty and cost in combining the operations and personnel of any future acquired businesses with our operations and personnel; impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership; and inability to retain key employees of any acquired businesses.

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Accordingly, although we cannot assure you that we will undertake or successfully complete any transactions of the nature described above, any transactions that we do complete may be subject to the foregoing or other risks and could have a material adverse effect on our business, results of operations and financial condition.

Our business involves the use of hazardous materials, and we and our third-party manufacturers and suppliers must comply with environmental laws and regulations, which can be expensive and restrict how we do business.

Our research and development activities and those of our third-party manufacturers and suppliers involve the controlled storage, use and disposal of hazardous materials, including components of our product and product candidates and other hazardous compounds. We and our manufacturers and suppliers are subject to laws and regulations governing the use, manufacture, storage, handling and disposal of these hazardous materials. In some cases, these hazardous materials and various wastes resulting from their use are stored at our and our manufacturers' facilities pending their use and disposal. We cannot eliminate the risk of contamination, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. We do not currently carry biological or hazardous waste insurance coverage. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance.

Significant disruptions of information technology systems or breaches of data security could materially adversely affect our business, results of operations and financial condition.

We are increasingly dependent on information technology systems and infrastructure to operate our business. In the ordinary course of our business, we collect, store and transmit large amounts of confidential information, including intellectual property, proprietary business information and personal information. It is critical that we do so in a secure manner to maintain the confidentiality and integrity of such confidential information. We have also outsourced elements of our information technology infrastructure, and as a result a number of third-party vendors may or could have access to our confidential information. The size and complexity of our information technology systems, and those of third-party vendors with whom we contract, make such systems potentially vulnerable to breakdown, malicious intrusion, security breaches and other cyber-attacks. In addition, the prevalent use of mobile devices that access confidential information increases the risk of data security breaches, which could lead to the loss of confidential information, trade secrets or other intellectual property. The costs to us to eliminate or alleviate network security problems, bugs, viruses, worms, malicious software programs and security vulnerabilities could be significant, and while we have implemented security measures to protect our data security and information technology systems, our efforts to address these problems may not be successful, and these problems could result in unexpected interruptions, delays, cessation of service and other harm to our business. Despite the extensive measures we may take to secure data and our information technology systems, a determined hacker or other bad actor may still breach these security measures and our information technology systems. Moreover, if a computer security breach affects our systems or results in the unauthorized release of personally identifiable information, our reputation could be materially damaged. In addition, such a breach may require notification to governmental agencies, the media or individuals pursuant to various federal and state privacy and security laws, if applicable, including the Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Clinical Health Act of 2009, and its implementing rules and regulations, as well as regulations promulgated by the Federal Trade Commission and state breach notification laws. We would also be exposed to a risk of loss or litigation and potential

liability, which could materially adversely affect our business, results of operations and financial condition.

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Business disruptions from the occurrence of a catastrophic disaster could cause damage to our facilities and equipment or that of our third-party manufacturers or suppliers.

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 our contract manufacturers and source suppliers of raw materials and critical services are also vulnerable to damage from other types of disasters, including fires, storms and other extreme weather conditions, floods, water shortages, power losses, telecommunications failures, outbreaks of disease and similar events. If such a disaster were to occur, our ability to continue our operations, including commercial sales and 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, and we may not be able to maintain insurance in the future at satisfactory rates or in adequate amounts.

Risks Related to Intellectual Property and Competition

If we are unable to protect adequately our proprietary technology, we may not be able to compete as effectively, and our business, results of operations and financial condition will be materially adversely affected.

Our success depends significantly on our ability to protect our proprietary technology from unauthorized use by third parties. We will be able to obtain such protection only to the extent our products are covered by valid and enforceable patents or trade secrets. Any non-confidential disclosure to or misappropriation by third parties of our confidential or proprietary information could enable competitors to quickly duplicate or surpass our technological achievements and erode our competitive position in the market. Where we elect to pursue patent protection on our proprietary technology, we file, prosecute and maintain patent applications covering certain aspects of our technology. Patent protection may not be available, however, for some of the drug product candidates we are developing. We are required to spend significant time and money obtaining, maintaining and enforcing our patent rights, designing around patents held by others and obtaining licenses to third-party patents or other proprietary rights that cover aspects of our product candidates.

The patent application process, also known as patent prosecution, is expensive and time consuming. It is possible that we or our current licensors, or any future licensors or licensees, may not be able to prepare, file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner or identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. If our current licensors, or any future licensors or licensees, are not fully cooperative or disagree with us regarding any aspect of the prosecution, maintenance or enforcement of the patent rights covering our product candidates where they have decision-making rights on such matters, our preferred approach may not be followed and the scope, strength, duration or other aspects of such patent rights could be compromised.

In addition, our patents and applications or those of our licensors may not be prosecuted and enforced in a manner consistent with the best interests of our business. Defects in form in the preparation or filing of our patents or patent applications or those of our licensors may adversely affect proper priority claims, inventorship, claim scope or patent term adjustments. As a result, the patent rights we depend upon to protect our technology may be held invalid or unenforceable or may be limited in scope. Moreover, we cannot assure you that all of the patent applications that we own or license will issue as patents or that, if issued, the claims of such patents will be held valid or enforceable or will have a scope that will be advantageous to us.

The rights granted to us under the issued patents, as well as those that may be granted on pending patent applications that we own or license, may not be of sufficient scope or strength to provide us with any meaningful protection or commercial advantage. In such case, competitors may be able to design around our patents or develop products that provide outcomes comparable to ours without infringing on our intellectual property rights. In addition, we may in the

future be subject to claims by our former employees or consultants asserting an ownership right in our patents or patent applications as a result of the work they performed on our behalf. Although we generally require all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to assign or grant similar rights to us under their inventions arising while working for us, we cannot be certain that we have executed such agreements with all who may have contributed to our inventions and intellectual property, nor can we be certain that our agreements with such parties will not be breached.

If any of our patents or those of our licensors are challenged, invalidated or legally circumvented by third parties, and if we do not own other enforceable patents protecting our products, competitors could market products and use processes that are substantially similar to, or superior to, ours, and our business will suffer. Any of these outcomes could impair our ability to succeed against competition from third parties and materially adversely affect our business, results of operations and financial condition.

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Our patents, even if issued, may not afford us the degree of protection we require to maintain a competitive advantage.

We own or license issued U.S. and foreign patents and pending U.S. and foreign patent applications related to certain of our drug product candidates and our other technologies. Evaluating the strength of patents covering our products candidates and other technologies in the biopharmaceutical field involves complex legal and scientific questions and can be highly uncertain. While we also rely on orphan drug exclusivity for PROCYSBI for commercial protection, the degree of patent protection we require may be unavailable or severely limited in some cases and may not adequately protect our products or permit us to gain or keep any competitive advantage. For example, the patent applications that we own or license may fail to result in issued patents in the United States or in other countries. Even if patents do issue on such patent applications, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. For example, U.S. patents can be challenged by any person before the U.S. Patent and Trademark Office ("USPTO") Patent Trial and Appeal Board ("PTAB"). Patents granted by the European Patent Office may be similarly opposed by any person within nine months from the publication of the grant. Similar proceedings are available in other jurisdictions, and in the United States, Europe and other jurisdictions, third parties can raise questions of validity with a patent office even before a patent has granted. See also the risk factor titled "Changes to the patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products."

Even if they are unchallenged, our patents and patent applications, if granted, may not adequately protect our product candidates or technology or prevent others from designing around our patent claims. For example, a third party may develop a competitive product that provides therapeutic benefits similar to one or more of our product candidates but that has a different composition that falls outside the scope of our patent protection. If the breadth or strength of protection provided by the patents and patent applications we own or license covering our product candidates is successfully challenged, then our ability to commercialize such product candidates could be adversely affected, and we may face unexpected competition that may materially adversely affect our business, results of operations and financial condition.

In addition, competitors may interfere with our success in obtaining and maintaining patent protection for our product candidates and technologies in a variety of ways. Competitors may claim that they invented the claimed invention prior to us or our licensors or may file patent applications before we or our licensors do. For example, because patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we or our licensors were the first to file any patent application related to a product candidate. Furthermore, if third parties have filed such patent applications, for patent applications filed before March 2013 in the United States an interference proceeding in the United States can be initiated by a third party to determine who was the first to invent any of the subject matter covered by the patent claims of our applications.

Competitors may also claim that we are infringing on their patents and that we therefore cannot develop or commercialize our product candidates or practice our technology. Competitors may also challenge our patents, if issued, by showing the USPTO or a court that the invention claimed was not novel, was obvious or is not valid for a number of other reasons. If the USPTO or a court agrees, we could lose some or all of our rights to the challenged patents.

Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Thus, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced. Without adequate and continuing patent protection for our product candidates and technologies, we may be open to competition from generic versions of such products and competitive versions of our technologies.

If we do not obtain, or if we lose, adequate patent protection for our product candidates, and if we do not have other regulatory exclusivity for such product candidates as described under the risk factor titled “If we fail to maintain orphan drug or other regulatory exclusivity for PROCYSBI or to obtain and maintain exclusivity for our orphan drug product candidates, our competitors may sell products to treat the same conditions, possibly at lower prices, and our revenues will be significantly reduced,” others may develop and commercialize products that are the same as, or similar to, our product candidates, which would adversely affect our business, results of operations and financial condition.

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We may in the future become involved in lawsuits to defend against third-party allegations of infringement or to protect or enforce our intellectual property, which could be expensive, time consuming and unsuccessful and have a material adverse effect on our business, results of operations and financial condition.

The drug product and biopharmaceutical industry has been characterized by frequent and extensive intellectual property litigation. Our competitors or other third-party patent holders may assert that our products are covered by their patents. Although we believe we have adequate defenses available if faced with any allegations that we infringe third-party patents, we cannot be certain that we do not infringe on those patents or that we will not infringe on patents granted in the future. Furthermore, because patent applications can take many years to issue and may be confidential for 18 months or more after filing, and because pending patent claims can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use or sale of our products. If a patent holder believes our drug product infringes on its patent, the patent holder may sue us even if we have received patent protection for our technology. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant product revenue and against whom our own patent portfolio may thus have no deterrent effect. If our products are found to infringe, we could be prevented from manufacturing or marketing those products.

In addition, competitors may infringe our patents or misappropriate or otherwise violate our intellectual property rights. To stop any such infringement or unauthorized use, litigation may be necessary. If we or one of our licensors were to initiate legal proceedings against a third party to enforce a patent, the defendant could seek to have the patent's validity reviewed through PTAB proceedings or counterclaim that our patent is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements for patent issuance, including lack of novelty, obviousness or enablement. Patents may be unenforceable if someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. The outcomes of proceedings involving assertions of invalidity and unenforceability are unpredictable. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability of our patents covering one of our product candidates or technologies, we would lose at least part, and perhaps all, of the patent protection on such product candidates or technologies. Such a loss of patent protection would materially adversely affect our business, results of operations and financial condition, particularly if we do not have other regulatory protection. Moreover, our competitors could counterclaim in any suit to enforce our patents that we infringe their intellectual property.

Third parties may initiate legal proceedings against us to challenge the validity or scope of our intellectual property rights or may allege an ownership right in our patents resulting from their past employment or consultancy with us. Many of our current and potential competitors have the ability to dedicate substantially greater resources to defend their intellectual property rights than we can. Accordingly, despite our efforts, we may not be able to prevent third parties from claiming an ownership interest in or infringing upon or misappropriating our intellectual property. Competing products may also be sold in other countries in which our patent coverage might not exist or be as strong. If we lose a foreign patent lawsuit alleging our infringement of a competitor's patents, we could be prevented from marketing our product candidates in one or more foreign countries.

Litigation related to infringement or misappropriation of a third parties' intellectual property rights, with or without merit, is unpredictable, is generally expensive and time consuming and can divert management's attention from our core business. If we do not prevail in any litigation in which we are alleged to have infringed or misappropriated intellectual property rights, a court could require us to pay substantial damages, treble damages and attorneys' fees and could prohibit us from using technologies essential to our product candidates, any of which would have a material adverse effect on our business, results of operations and financial condition. If patents asserted against us are upheld as valid and enforceable and we are found to infringe them, we could be prevented from selling our product candidates or technologies unless we can obtain licenses to use the technology or ideas covered by such patents. We do not know whether any necessary licenses would be available to us on satisfactory terms, if at all. If we cannot obtain such

licenses, we could be forced to design around the infringed patents at additional cost or to abandon the infringing product candidate or technology altogether. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the market price of our common stock. As a result, our ability to grow our business and compete in the market may be harmed.

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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 product candidates derived from these collaborations. 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. Any such disputes may cause our competitive position to be adversely affected and may materially adversely affect our business, results of operations and financial condition.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In addition, periodic maintenance fees on issued patents often must be paid to the USPTO and foreign patent agencies over the lifetime of the patent. While an unintentional lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates or technologies, we may not be able to prevent a competitor from marketing products that are the same as or similar to our products, which would have a material adverse effect on our business, results of operations and financial condition, particularly if we do not have other regulatory protection for our products.

We may not be able to effectively enforce our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on our product candidates and technologies in all countries throughout the world would be prohibitively expensive. The requirements for patentability may differ in certain countries, particularly in developing countries. Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. In addition, laws of some countries outside of the United States do not afford intellectual property protection to the same extent as the laws of the United States. Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of some countries, particularly developing countries, do not favor the enforcement of patents and other intellectual property rights. This could make it difficult for us to stop the infringement of our patents or the misappropriation of our other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States. In addition, competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, if our ability to enforce our patents to stop infringing activities is inadequate. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing. See also the risk factor titled “Our success depends on our ability to manage our projected growth.”

Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business. Furthermore, while we intend to protect our intellectual property rights in major markets for our products, there can be no assurance that we will be able to

initiate or maintain similar efforts in all jurisdictions in which we may wish to market our products. In addition, our efforts to protect our intellectual property rights in such countries may be inadequate.

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Changes to the patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biopharmaceutical companies, our success is heavily dependent on our having valid and enforceable intellectual property rights, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Recent patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act (the "America Invents Act"), which became effective on September 16, 2012, could increase those uncertainties and costs. The America Invents Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. For example, the America Invents Act enacted proceedings involving post-issuance patent review procedures conducted before the PTAB, including inter partes review ("IPR"). The IPR process permits third parties to challenge the validity of a patent on the grounds that it was anticipated or made obvious by prior art, and generic drug manufactures and entities associated with hedge funds have recently begun challenging biopharmaceutical patents with increased frequency based on prior art through the IPR process. Prior art could render our patents or those of our licensors invalid, and the availability of the IPR process as a lower-cost alternative to litigation and faster method for challenging patents could therefore increase the likelihood that our patents or those of our licensors will be challenged and potentially rendered invalid. Moreover, if such challenges occur with respect to our UCSD licensed patents, UCSD has the right to control the defense of such proceedings.

In addition, the America Invents Act has transformed the U.S. patent system into a "first to file" system. The first-to-file provisions, however, only became effective on March 16, 2013. Accordingly, it is not yet clear what, if any, impact the America Invents Act will have on the operation of our business. However, the America Invents Act and its implementation could make it more difficult to obtain patent protection for our inventions and increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could materially adversely affect our business, financial condition and results of operations.

The U.S. Supreme Court has ruled on several patent cases in recent years, narrowing the scope of patent protection available in certain circumstances and weakening the rights of patent owners in certain situations. In addition, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could affect our ability to obtain patent protection for our proprietary technology or our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

If we are limited in our ability to utilize acquired or licensed technologies, or if we lose our rights to critical in-licensed technology, we may be unable to successfully develop, out-license, market and sell our products, which could prevent or delay new product introductions.

We have acquired and licensed certain proprietary technologies and plan to further license and acquire various patents and proprietary technologies owned by other parties. The agreements in place are critical to our product development programs. For example, most of our patent portfolio pertaining to PROCYSBI and RP103 for cystinosis and other therapeutic indications has been licensed from academic institutions. Our license agreements with these institutions include termination clauses that permit the licensor to terminate our license under certain circumstances, including if we materially breach our obligations under the applicable agreement. If one or more of our licenses is terminated, we would have no further right to use or exploit the patents, know-how and other intellectual property rights licensed to us under the agreement, which could adversely affect our ability to market PROCYSBI or continue our development

programs of RP103 in other clinical indications.

Our business strategy depends on the successful development of licensed and acquired technologies into commercial products. Therefore, any limitations on our ability to utilize these technologies may impair our ability to develop, out-license or market and sell our product candidates, could delay new product introductions and could adversely affect our reputation, any of which could have a material adverse effect on our business, results of operations and financial condition.

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If we are unable to protect the confidentiality of our trade secrets, our competitive position may be harmed, and our business, results of operations and financial condition will be materially adversely affected.

In addition to patent protection, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. Our trade secrets may not be adequately protected, however. We have taken steps to protect our trade secrets and proprietary information, including entering into confidentiality agreements and intellectual property assignment agreements with our employees, consultants, advisors and corporate and educational institution partners. Nevertheless, 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, whether willful or unintentional, 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. In addition, we may not be able to obtain adequate remedies for any such breaches. Enforcing a claim that someone 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 United States may be less willing to protect trade secrets. Our competitors may independently develop equivalent knowledge, methods and know-how. Competitors could purchase our products and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe or misappropriate our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us.

We may be subject to damages resulting from claims that we or our employees have wrongfully used or disclosed alleged trade secrets of our competitors or are in breach of non-competition or non-solicitation agreements with our competitors.

We could in the future be subject to claims that we or our employees have inadvertently or otherwise used or disclosed alleged trade secrets or other proprietary information of former employers or competitors. Although we try to ensure that our employees and consultants do not use the intellectual property, proprietary information, know-how or trade secrets of others in their work for us, we may in the future be subject to claims that we caused an employee to breach the terms of his or her non-competition or non-solicitation agreement or that we or these individuals have, inadvertently or otherwise, used or disclosed the alleged trade secrets or other proprietary information of a former employer or competitor in breach of their obligations to that employer or competitor. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial costs and could be a distraction to management. If our defenses to these claims fail, in addition to requiring us to pay monetary damages, a court could prohibit us from using technologies or features that are essential to our products, if such technologies or features are found to incorporate or be derived from the trade secrets or other proprietary information of the former employers. An inability to incorporate such technologies or features would have a material adverse effect on our business, and may prevent us from successfully commercializing our products. In addition, we may lose the right to practice valuable intellectual property rights or personnel as a result of such claims. Moreover, any such litigation or the threat thereof may adversely affect our ability to hire employees or contract with independent sales representatives. A loss of key personnel or their work product could hamper or prevent our ability to commercialize our products. Any of these events, or a combination thereof, could have a material adverse effect on our business, results of operations and financial condition.

Risks Related to Our Financial Position and Capital Requirements

Our commercial operations and clinical development programs will require substantial future funding which will affect our operational and financial condition.

Our commercial sales programs for PROCYSBI and any future approved products and our product development programs will require substantial additional capital, arising from costs incurred to:

- conduct research, preclinical testing and human studies and clinical trials; develop and submit regulatory submissions for marketing approvals;
- develop and submit regulatory submissions for marketing approvals;
- establish or contract for pilot scale and commercial scale manufacturing processes and facilities;
- obtain adequate reimbursement for our products;
- market and distribute PROCYSBI and any future approved products; and
- establish, develop and maintain quality control, manufacturing, regulatory, medical, distribution, marketing, sales, finance and administrative capabilities to support these programs.

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We base our outlook regarding the need for funds on many uncertain variables. Such uncertainties include the success of our commercial sales of PROCYSBI in the United States, the EEA and any additional markets; the success of our efforts to commercialize any future approved products; the scope and results of our research initiatives, preclinical testing and human clinical trials; regulatory approvals; the timing of events outside our direct control, such as competing technological and market developments, negotiations with third-party payors and potential strategic partners; and other factors. In addition, certain product programs may require collaborative agreements with corporate partners with greater financial and organizational resources than we have. Such agreements may require substantial time to complete and may not be available in the time frame desired or with acceptable financial terms, if at all. Any of these factors may significantly change the timing and amount of our cash requirements as they determine such one-time events as the receipt or payment of milestone-based and other payments.

If we fail to raise additional financing when needed, we may have to delay or terminate some or all of our research and development programs, scale back our operations and/or reduce our commercial finance and administrative expenses to sustainable levels, which would have a material adverse effect on our business, results of operating and financial condition.

While we believe that, based on current operating plan assumptions, our cash and cash equivalents will be sufficient to fund operations through at least the second half of 2017, we will need to sell equity or debt securities to raise additional funds to support, among other things, our development and commercialization programs. The sale of additional equity securities or convertible debt securities will result in additional dilution to our stockholders, and newly issued securities may have rights, preferences and privileges senior to those of holders of our common stock. Additional financing may not be available on a timely basis, in amounts or on terms satisfactory to us, or at all. We may be unable to raise additional capital due to a variety of factors, including our financial condition, the status of our research and development programs, the status of regulatory reviews for marketing approvals, the status of our commercialization activities, sales of PROCYSBI in existing and additional markets and the general condition of the financial markets. If we fail to raise additional financing when needed, we may have to delay, partner, or terminate some or all of our research and development programs, scale back our operations and/or reduce our commercial expenses. If such actions are required, our business, results of operations and financial condition will be adversely affected, and the market value of our common stock may significantly decline.

Our loan agreement with HC Royalty and outstanding convertible senior notes contain a number of restrictive covenants and other provisions, which, if violated, could result in the acceleration of the payment terms of our outstanding indebtedness, which in turn could have a material adverse effect on our business, results of operations and financial condition.

In December 2012, we entered into a loan agreement with HC Royalty, as lender, which we refer to as the “HC Royalty Loan Agreement.” Under the HC Royalty Loan Agreement, we agreed to borrow $50.0 million in two $25.0 million tranches. We drew down the first tranche in the amount of $25.0 million in December 2012 and the second tranche of $25.0 million in May 2013 when we achieved the milestone of U.S. approval of PROCYSBI. In July 2014, we entered into an amendment and restatement of the original HC Royalty Loan Agreement and borrowed from HC Royalty a third, $10.0 million tranche under the loan facility. Also in July 2014, we issued $60.0 million aggregate principal amount of 8.0% convertible senior notes due 2019 to HC Royalty and other purchasers.

The HC Royalty Loan Agreement includes a number of affirmative and negative covenants, including requirements to use commercially reasonable efforts to exploit PROCYSBI and RP103 in specific markets and to comply with applicable laws, and additional restrictions on mergers, sales of assets, the incurrence of liens, the incurrence of additional indebtedness and transactions with our affiliates, among other requirements. The convertible senior notes also include a number of affirmative and negative covenants, including our obligation to offer to repurchase the notes upon a change of control of our company, limitations on the incurrence of additional indebtedness, registration rights for the holders of the notes and other requirements.

The performance of our obligations under the HC Royalty Loan Agreement is secured by our grant of a security interest to HC Royalty in substantially all of our assets and the assets of our domestic subsidiaries and a pledge of stock of certain of our domestic subsidiaries. Our failure to comply with the terms of the HC Royalty Loan Agreement, the convertible senior notes and related documents could result in an event of default. A change of control of our company, an uncured material adverse effect on our company and certain other specified events could also constitute an event of default under the agreements. In the event of an event of default that is not cured or waived, the payment of all of our indebtedness to HC Royalty and interest thereon and the repayment of the convertible senior notes could accelerate. Under the terms of the security agreement, an event of default could also enable the lender to take possession of, foreclose on, sell, assign or grant a license to use our pledged collateral and to assign and transfer the pledged stock of certain of our subsidiaries. An event of default, a material adverse effect or a change of control would also trigger a prepayment penalty under the HC Royalty Loan Agreement, which would require us to pay a substantially higher amount due than the current balance of our loan.

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Any of the events described above, or a combination thereof, could have a material adverse effect on our financial condition and results of operations.

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

The required payments of principal and interest on our indebtedness under the HC Royalty Loan Agreement and convertible senior notes may require a substantial portion, or all, of our available cash to be dedicated to the service of these debt obligations. Both the HC Royalty Loan Agreement and the convertible senior notes bear interest at an annual fixed rate of 8.0%. The HC Royalty Loan Agreement also bears a synthetic royalty based on our net revenues from PROCYSBI and other future-approved products in a calendar year. This royalty and the interest under the HC Royalty Loan Agreement and the convertible senior notes are payable quarterly. Principal payments under the HC Royalty Loan Agreement will become due beginning in June 2015. The convertible senior notes will mature on August 1, 2019, unless earlier converted, redeemed or repurchased.

There can be no assurance that our business will generate sufficient cash flow or that we will have capital resources in an amount sufficient to enable us to pay our indebtedness to HC Royalty or the holders of the convertible senior notes. Our debt obligations may also limit our flexibility to plan for or react to changes in our business and industry and place us at a competitive disadvantage compared to competitors with superior financial resources including less debt. If our cash flows and capital resources are insufficient to fund these debt service obligations, we may be forced to reduce or delay product development, sales and marketing and capital and other expenditures. We may also be forced to restructure our indebtedness or raise additional capital through the issuance of equity or debt instruments, and there can be no assurance that we will be able to refinance any of our indebtedness or raise additional capital on a timely basis, in sufficient amounts, on satisfactory terms or at all. The terms of the HC Royalty Loan Agreement and convertible senior notes may also limit our ability to pursue any of these financing alternatives, and these alternatives nonetheless may not enable us to meet our scheduled debt service obligations.

Failure to meet our debt service obligations may result in an event of default under the HC Royalty Loan Agreement, which would permit the lender to accelerate the payment of all of our indebtedness to HC Royalty and interest thereon. An event of default could also enable the lender to take possession of, foreclose on, sell, assign or grant a license to use our pledged collateral and to assign and transfer the pledged stock of certain of our subsidiaries. An event of default, a material adverse effect or a change of control would also trigger a prepayment penalty under the HC Royalty Loan Agreement, which would require us to pay a substantially higher amount than the current balance of our loan.

Failure to meet our debt service obligations may also result in an event of default under the convertible senior notes, which would permit the holders to accelerate the payment of the outstanding principal amount of the notes and interest thereon and require us to pay a repayment premium and higher interest. A change of control would also trigger an obligation to repurchase the convertible senior notes.

Any of the events described above, or a combination thereof, could have a material adverse effect on our business, financial condition and results of operations.

Our ability to use our net operating losses to offset future taxable income may be subject to certain limitations.

In general, under Section 382 of the Internal Revenue Code of 1986, as amended, a corporation that undergoes an “ownership change” is subject to limitations on its ability to utilize its pre-change net operating loss carryforwards to offset future taxable income. Our existing net operating loss carryforwards may be subject to limitations arising from previous ownership changes, and if we undergo an ownership change in the future, our ability to utilize our net operating loss carryforwards could be further limited by Section 382. Future changes in our stock ownership, some of which are outside of our control, could result in an ownership change. Furthermore, we may be unable to use a

substantial part of our net operating loss carryforwards if we do not attain profitability in an amount sufficient to utilize such losses.

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Risks Related to Our Common Stock

Our stock price has been volatile and may continue to be volatile in the future, and our stockholders may not be able to resell shares of our common stock at or above the prices that they paid. The trading volume in our common stock may be relatively small.

Our common stock is quoted on The NASDAQ Global Market. The trading price of our common stock has been and may continue to be volatile. During the 52-week period ended March 31, 2015, our average daily trading volume was approximately 777,372 shares and the closing sales price per share of our common stock on The NASDAQ Global Market ranged from $7.12 to $12.20. Our operating performance, both financial and in the development of approved products, significantly affects the market price of our common stock. A number of factors may affect the market price of our common stock, including:

- the success of our testing and clinical trials and those of others with products similar or related to our products;
- announcements regarding regulatory approvals or approved label indications and patient populations or changes or delays in the regulatory review process;
- unexpected difficulties in commercialization or lower than expected sales;
- lower than expected pricing and reimbursement levels, or no reimbursement at all, for our products in various markets;
- actions taken by regulatory agencies with respect to our clinical trials, manufacturing supply chain or sales and marketing activities;
- changes in our relationships with manufacturers, suppliers or collaborators, or our inability to supply enough product to meet demand;
- announcements of new products or innovations by us or our competitors and announcements concerning our competitors or our industry in general;
- our ability to obtain additional funding;
- changes or developments in applicable laws or regulations;
- any intellectual property infringement actions in which we may become involved;
- sales and profitability;
- announcements of significant acquisitions, strategic partnerships, joint ventures, or capital commitments by us, our strategic collaboration partners or our competitors;
- our ability to manage our projected growth;
- actual or anticipated fluctuations in our results of operations;
- changes in financial estimates or recommendations by securities analysts or their ceasing to publish research or reports about our business;

- the trading volume of our common stock;

- general economic and market conditions and overall fluctuations in the U.S. equity markets;

- the appeal and current level of investor interest in the biotechnology/biopharmaceutical capital market sector and in companies in general with business, research strategies and product development pipelines which are similar to us; and

- the loss of any of our key scientific or management personnel.

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For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory, market launch and commercialization goals, which we sometimes refer to as milestones. These milestones include the completion of reimbursement and pricing negotiations and commercial launches in additional territories, commencement or completion of scientific studies and clinical trials and the submission of regulatory filings. We also prepare estimates of future financial results for planning and budget purposes. From time to time, we may publicly announce the estimated timing of some of these milestones and provide guidance regarding financial results and other metrics. All of these projections will be based on a variety of assumptions. The actual timing of these milestones and actual financial results can vary dramatically compared to our estimates for a number of reasons, including those set forth above, in many cases for reasons beyond our control. If we do not meet the milestones, financial guidance or other expectations as publicly announced or as projected by various security analysts who follow our company, our stockholders or potential stockholders may lose confidence in our ability to meet overall objectives and our financial planning capabilities, and as a result, the market price of our common stock may decline.

In addition, The NASDAQ Global Market has, from time to time, experienced extreme price and trading volume fluctuations. The market prices of securities of pharmaceutical, biotechnology and other life sciences companies in a comparable stage to ours historically have been particularly volatile, and trading volume in such securities and our common stock has often been relatively small. Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated or disproportionate to the operating performance of individual companies. During certain periods, the favor of certain industry segments, such as the biotechnology segment, may also be volatile. These changes may affect in particular the market price of our common stock and the stock prices of companies such as ours that do not have conventional measures of financial and business health such as sales, earnings, profitability and related measures. These broad market fluctuations, during which our industry and companies at our stage may experience a stronger degree of market sensitivity, will adversely affect the market price of our common stock. In the past, following periods of volatility in the market resulting in substantial declines 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 and reputation and materially adversely affect our business, financial condition and results of operations.

Future sales or issuances of our common stock or other securities in the public market, including shares issuable upon conversion of our convertible senior notes, or the perception of such future sales or issuances, could lead to a decline in the market price of our common stock.

Any issuance of shares of our common stock or other securities, including for the purposes of raising capital to fund our operations, acquisitions and the expansion of our business, will have a dilutive effect on our existing stockholders. In addition, any issuance or the perceived market risk associated with any possible issuance could cause the market price of our common stock to decline. Subsequent sales of our common stock in the open market, exercises of our currently outstanding stock options, conversion of our convertible senior notes and the subsequent sale of the shares acquired thereunder or any other issuance by us of shares of our common stock or other securities could also have an adverse effect on the market price of our common stock. If the market price of our common stock declines, it will be more difficult for us to raise additional capital, or we may be unable to raise additional capital at all.

In addition, we have in the past and may in the future grant rights to some of our stockholders that require us to register the resale of shares or our common stock on behalf of these stockholders and/or to facilitate offerings of shares of our common stock held by these stockholders, including in connection with potential future acquisitions of additional products, product candidates or companies. If holders of such registration rights sell a large number of shares of our common stock, the sale could cause the market price of our common stock to decline. We have also filed registration statements to register the sale of our shares of our common stock reserved for issuance under our equity incentive plans and our employee stock purchase plan and intend to file additional registration statements to register any shares added to the reserves under these plans.

In July 2014, we issued $60.0 million aggregate principal amount of 8.0% convertible senior notes due 2019. The convertible senior notes are convertible at the option of the holder at an initial conversion rate of approximately 57.14 shares of common stock per $1,000 principal amount of convertible senior notes, which is equivalent to an initial conversion price of $17.50, and is subject to adjustment upon certain events and conditions. In addition, we may redeem for cash or require holders to convert the convertible senior notes into shares of common stock if the price of the common stock is at or above 175% of the applicable conversion price over a period of 30 consecutive trading days. The note purchase agreement governing the convertible senior notes provides the holders with registration rights for the shares issued upon conversion of their convertible senior notes subject to certain conditions, and we have filed a registration statement to register the resale of the shares of common stock issuable upon conversion of the convertible senior notes. We may be required to pay increased interest on the convertible senior notes if we do not comply with the registration rights provisions of the note purchase agreement. A substantial number of shares of our common stock are reserved for issuance upon conversion of the convertible senior notes. The issuance of shares of our common stock upon conversion of the convertible senior notes would dilute the ownership interest of our common stockholders and may materially adversely affect the market price of our common stock and impair our ability to raise capital through the sale of additional equity securities.

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In connection with other collaborations, joint ventures, license agreements, acquisitions 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 common stockholders.

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

We have not declared or paid any cash dividends on our common stock since our inception. We anticipate that we will retain our earnings to support our operations and to finance the growth and development of our business and do not expect to pay cash dividends in the foreseeable future. Our loan agreement with HC Royalty prohibits us from paying cash dividends. As a result, the success of an investment in our common stock will depend upon any future appreciation in the market price of our common stock. There can be no guarantee that the market price of our common stock will appreciate or that it will not depreciate. Investors seeking dividend income should not invest in our common stock.

Anti-takeover provisions under Delaware law, in our stockholder rights plan and in our Certificate of Incorporation and Bylaws, as amended, 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.

Our Certificate of Incorporation and Bylaws, as amended, contain provisions that could significantly reduce the value of our shares to a potential acquirer or delay or prevent changes in control or changes in our management without the consent of our Board of Directors. The provisions in our charter documents include the following:

- the right of our Board of Directors to elect a director to fill a vacancy created by the expansion of the Board of Directors or the resignation, death or removal of a director, which prevents stockholders from being able to fill vacancies on our Board of Directors;
- the required approval of at least 66 2/3% of the shares entitled to vote to remove a director without cause;
- the ability of our Board of Directors to authorize the issuance of shares of preferred stock and to determine the price and other terms of those shares, including preferences, dividend rights and voting rights, which may be superior to those of the common stock, without stockholder approval, which could be used to significantly dilute the ownership of a hostile acquiror;
- the ability of our Board of Directors to alter our bylaws without obtaining stockholder approval;
- the required approval of at least 66 2/3% of the shares entitled to vote at an election of directors to adopt, amend or repeal our bylaws;
- a prohibition on stockholder action by written consent, which forces stockholder action to be taken at an annual or special meeting of our stockholders;
- the requirement that a special meeting of stockholders may be called only by the Chairman of the Board of Directors, the chief executive officer or the Board of Directors, which may delay the ability of our stockholders to force consideration of a proposal or to take action, including the removal of directors; and
- advance notice procedures that stockholders must comply with in order to nominate candidates to our Board of Directors or to propose matters to be acted upon at a stockholders' meeting, which may discourage or deter a potential acquiror from conducting a solicitation of proxies to elect the acquiror's own slate of directors or otherwise attempting

to obtain control of us.

We are also subject to the anti-takeover provisions contained in Section 203 of the Delaware General Corporation Law. Under Section 203, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other exceptions, the Board of Directors has approved the transaction.

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We are also 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 for and less desirable to the potential acquirer.

Our Board of Directors may use the provisions described above to prevent changes in the management and control of our company. These provisions could limit the price that investors might be willing to pay in the future for shares of our common stock. These provisions would apply even if we were to receive an offer that some stockholders may consider beneficial. Also, under applicable Delaware law, our Board of Directors may adopt additional anti-takeover measures in the future.

Provisions of the note purchase agreement governing our convertible senior notes may discourage a takeover, which could cause the market price of our common stock to decline.

The repurchase rights and related repurchase premium provided in our convertible senior notes triggered by the occurrence of a change of control may discourage, delay or prevent a third party from acquiring us, even if doing so would benefit our stockholders. In turn, this could cause the market price of our common stock to decline.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS.

None.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES.

None.

ITEM 4. MINE SAFETY DISCLOSURES.

None.

ITEM 5. OTHER INFORMATION.

None.

ITEM 6. EXHIBITS

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which are incorporated herein by reference.

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SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

RAPTOR PHARMACEUTICAL CORP.

Date: May 7, 2015 By: /s/ Julie A. Smith
Julie A. Smith
Chief Executive Officer and Director
(Principal Executive Officer)

Date: May 7, 2015 By: /s/ Michael P. Smith
Michael P. Smith
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

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Exhibit Index

Exhibit Number	Exhibit Description	Incorporated by Reference			Filed Herewith
		Form	Date	Number	
10.1	Executive Employment Agreement, dated as of January 2, 2015, by and between Michael Smith and Raptor Pharmaceutical Corp.	8-K	1/7/2015	10.1	
10.2	Executive Employment Agreement, dated as of January 2, 2015, by and between Krishna Polu and Raptor Pharmaceutical Corp.	10-K	3/2/2015	10.48	
10.3	Form of Restricted Stock Unit Agreement under Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan				X
31.1	Certification of Julie Anne Smith, Chief Executive Officer and Director				X
31.2	Certification of Michael P. Smith, Chief Financial Officer, Secretary and Treasurer				X
32.1*	Certification of Julie Anne Smith, Chief Executive Officer and Director, and Michael P. Smith, Chief Financial Officer, Secretary and Treasurer				X
101	The following materials from the Raptor Pharmaceutical Corp. Quarterly Report on Form 10-Q for the quarter ended March 31, 2015, formatted in Extensible Business Reporting Language (XBRL): (i) the Condensed Consolidated Balance Sheets; (ii) the Condensed Consolidated Statements of Operations and Comprehensive Loss; (iii) the Condensed Consolidated Statements of Cash Flows; and (iv) related notes, tagged as blocks of text.				X

* In accordance with Item 601(b)(32)(ii) of Regulation S-K, this exhibit shall not be deemed “filed” for the purposes of Section 18 of the Exchange Act or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act.