Raptor Pharmaceutical Corp
Form 10-Q
May 09, 2014

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

FORM 10-Q

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

For the quarterly period ended March 31, 2014

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 or other jurisdiction of incorporation or organization) | (I.R.S. Employer Identification No.) |

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

(415) 408-6200
(Registrant's telephone number, including area code)

_____
(Former name, former address and former fiscal year, if changed since last report)

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 þ 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 þ 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 þ
Non-accelerated filer ¨ (Do not check if a smaller reporting company) Smaller reporting company ¨

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

There were 62,616,859 shares of the registrant's common stock, par value $0.001, outstanding as of April 30, 2014.

RAPTOR PHARMACEUTICAL CORP.

FORM 10-Q FOR THE THREE MONTHS ENDED MARCH 31, 2014

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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, 2014 | December 31, 2013 |
|---|---|---|
| ASSETS | | |
| Current assets: | | |
| Cash and cash equivalents | $68,075 | $83,052 |
| Restricted cash | 1,030 | 500 |
| Accounts receivable | 7,079 | 6,181 |
| Inventories | 6,363 | 3,000 |
| Prepaid expenses and other | 2,962 | 3,566 |
| Total current assets | 85,509 | 96,299 |
| | | |
| Intangible assets, net | 3,153 | 3,213 |
| Goodwill | 3,275 | 3,275 |
| Fixed assets, net | 1,880 | 1,810 |
| Other assets | 4,230 | 4,129 |
| | | |
| Total assets | $98,047 | $108,726 |
| LIABILITIES AND STOCKHOLDERS' EQUITY | | |
| Liabilities | | |
| Current liabilities: | | |
| Accounts payable | $2,179 | $5,264 |
| Accrued liabilities | 13,245 | 12,767 |
| Common stock warrant liability | 726 | 7,066 |
| Deferred revenue | 4,985 | 4,698 |
| Deferred rent | 166 | 302 |
| Capital lease liability – current | 18 | 18 |
| Total current liabilities | 21,319 | 30,115 |
| | | |
| Note payable | 50,000 | 50,000 |
| Capital lease liability – long-term | 36 | 41 |
| Total liabilities | 71,355 | 80,156 |
| | | |
| Commitments and contingencies | | |
| Stockholders' equity: | | |
| Preferred stock, $0.001 par value per share, 15,000,000 shares authorized, zero shares issued and outstanding | 0 | 0 |
| Common stock, $0.001 par value per share, 150,000,000 shares authorized 62,598,859 and 61,614,576 shares issued and outstanding at March 31, 2014 and December 31, 2013, | 63 | 62 |

| | | |
|---|---|---|
| respectively | | |
| Additional paid-in capital | 247,148 | 234,286 |
| Accumulated other comprehensive loss | (273) | (423) |
| Accumulated deficit | (220,246) | (205,355) |
| Total stockholders' equity | 26,692 | 28,570 |
| | | |
| Total liabilities and stockholders' equity | $98,047 | $108,726 |

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

Raptor Pharmaceutical Corp.
Condensed Consolidated Statements of Operations
(Unaudited)
(In thousands, except shares and per share data)

| | For the three months ended March 31, | |
|---|---|---|
| | 2014 | 2013 |
| Revenues | $12,134 | $0 |
| Cost of sales | 1,314 | 0 |
| Gross profit | 10,820 | 0 |
| Operating expenses: | | |
| Research and development | 9,547 | 8,412 |
| Selling, general and administrative | 12,064 | 7,863 |
| Total operating expenses | 21,611 | 16,275 |
| Loss from operations | (10,791) | (16,275) |
| Interest income | 31 | 155 |
| Interest expense | (2,979) | (726) |
| Foreign currency transaction gain (loss) | 17 | (34) |
| Loss on short-term investments | 0 | (107) |
| Adjustment to fair value of common stock warrants | (1,163) | 1,060 |
| Net loss before provision for income taxes | (14,885) | (15,927) |
| Provision for income taxes | (6) | 0 |
| Net loss | $(14,891) | $(15,927) |
| Net loss per share: | | |
| Basic and diluted | $(0.24) | $(0.30) |
| Weighted-average shares outstanding used to compute earnings per share: | | |
| Basic and diluted | 62,118,796 | 53,713,037 |

See accompanying notes to condensed consolidated financial statements.

Raptor Pharmaceutical Corp.
Condensed Consolidated Statements of Comprehensive Loss
(Unaudited)
(In thousands)

| | For the three months ended March 31, | |
|---|---|---|
| | 2014 | 2013 |
| Net loss | $(14,891) | $(15,927) |
| Other comprehensive loss: | | |
| Foreign currency translation gain (loss) | 150 | (88) |
| Comprehensive loss | $(14,741) | $(16,015) |

See accompanying notes to condensed consolidated financial statements.

Raptor Pharmaceutical Corp.
Condensed Consolidated Statements of Cash Flows
(Unaudited)
(In thousands)

| | For the three months ended March 31, | |
|---|---|---|
| | 2014 | 2013 |
| Cash flows from operating activities: | | |
| Net loss | $(14,891) | $(15,927) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Stock-based compensation expense | 2,375 | 1,752 |
| Fair value adjustment of common stock warrants | 1,163 | (1,060) |
| Amortization of intangible assets | 60 | 36 |
| Depreciation of fixed assets | 117 | 36 |
| Loss on short-term investments | 0 | 107 |
| Amortization of debt issuance costs | 148 | 54 |
| | | |
| Changes in assets and liabilities: | | |
| Accounts receivable | (898) | 0 |
| Inventories | (3,363) | 0 |
| Prepaid expenses and other | 605 | 193 |
| Deposits | (221) | (194) |
| Accounts payable | (3,085) | (1,883) |
| Accrued liabilities | 478 | 2,414 |
| Deferred revenue | 287 | 0 |
| Deferred rent | (136) | (6) |
| Net cash used in operating activities | (17,361) | (14,478) |
| | | |
| Cash flows from investing activities: | | |
| Purchase of fixed assets | (187) | (112) |
| Change in restricted cash | (530) | (50) |
| Purchase of short-term investments | 0 | (134) |
| Net cash used in investing activities | (717) | (296) |
| | | |
| Cash flows from financing activities: | | |
| Proceeds from the sale of common stock under an ATM agreement | 0 | 13,444 |
| Proceeds from the exercise of common stock warrants | 1,826 | 1,845 |
| Proceeds from the exercise of common stock options | 1,158 | 7 |
| Fundraising costs | 0 | (495) |
| Debt issuance costs | 0 | (3) |
| Offering costs | (29) | 40 |
| Payments on capital lease | (4) | (1) |
| Net cash provided by financing activities | 2,951 | 14,837 |
| | | |
| Effect of exchange rates on cash and cash equivalents | 150 | (88) |
| | | |
| Net decrease in cash and cash equivalents | (14,977) | (25) |
| Cash and cash equivalents, beginning of period | 83,052 | 36,313 |

| | | |
|---|---|---|
| Cash and cash equivalents, end of period | $68,075 | $36,288 |
| | | |
| Supplemental cash flow information: | | |
| Interest paid | $2,600 | $672 |
| Income taxes paid | $46 | $0 |
| Supplemental disclosure of non-cash financing activities: | | |
| Fair value of warrant liability reclassified to equity upon exercise | $7,502 | $2,636 |

See accompanying notes to condensed consolidated financial statements.

RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
March 31, 2014
(In thousands, except share and per share data)

## 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, 2013 has been derived from our 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, 2013.

Raptor is a biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases. The Company's first product, PROCYSBI® (cysteamine bitartrate) delayed-release capsules ("PROCYSBI"), received marketing approval from the U.S. Food and Drug Administration ("FDA"), on April 30, 2013 for the management of nephropathic cystinosis in adults and children six years and older. The European equivalent, PROCYSBI® gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate), received marketing authorization on September 6, 2013 from the European Commission ("EC"), as an orphan medicinal product for the management of proven nephropathic cystinosis for marketing in the European Union ("EU"). PROCYSBI received 7 years and 10 years of market exclusivity as an orphan drug in the U.S. and the EU, respectively. The Company commenced commercial sales of PROCYSBI in the U.S. in mid-June 2013 and in Germany 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 U.S. for the management of nephropathic cystinosis in adults and children six years and older and in the EU for the management of nephropathic cystinosis.

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

The Company is subject to a number of risks, including: the level of commercial sales of PROCYSBI in the U.S. and Germany; the ability to successfully launch PROCYSBI in Germany and the rest of the EU and in other international markets; the uncertainty of whether the Company's research and development efforts will result in expanded indications for PROCYSBI or additional commercial products; competition from larger organizations; reliance on licensing the proprietary technology of others; dependence on key personnel; uncertain patent protection; and the need to raise capital through equity and/or debt financings. Funding may not be available when needed if at all or on terms acceptable to 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 condensed consolidated financial statements include the accounts of the Company's direct and indirect wholly owned subsidiaries, Raptor Pharmaceuticals Inc. and Raptor European Products, LLC, such subsidiaries incorporated in Delaware on August 1, 2007, and February 14, 2012, respectively, and Raptor Pharmaceuticals Europe B.V., Raptor Pharmaceuticals France SAS, Raptor Pharmaceuticals Germany GmbH and RPTP European Holdings C.V., domiciled in the Netherlands on December 15, 2009, in France on October 30, 2012, in Germany on October 16, 2013 and in the Cayman Islands on February 16, 2012, respectively. All inter-company accounts have been eliminated.

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 condensed consolidated financial statements and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.

Functional Currency

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

Cash and Cash Equivalents

The Company considers all highly liquid investments with original maturities of three months or less, when purchased, to be cash equivalents. The Company maintains cash and cash equivalents, which consist principally of money market funds with high credit quality financial institutions. Such amounts exceed Federal Deposit Insurance Corporation insurance limits. Restricted cash represents compensating balances required by the Company's U.S. and European banks as collateral for credit cards and for access to a value-added tax deferral program. As of March 31, 2014, the Company had $68.1 million in cash and cash equivalents, of which $5.8 million was held by its foreign subsidiaries.

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.

Revenue Recognition and Accounts Receivable

The Company recognizes revenue in accordance with the Financial Accounting Standards Board ("FASB") ASC 605, Revenue Recognition, when the following criteria have been met: persuasive evidence of an arrangement exists; delivery has occurred and risk of loss has passed; the seller's price to the buyer is fixed or determinable and collectability is reasonably assured. The Company determines that persuasive evidence of an arrangement exists based on written contracts that define the terms of the arrangements. Pursuant to the contract terms, the Company determines when title to products and associated risk of loss has passed 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.

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. Prior 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. Revenue is recognized once the product has been shipped by the specialty pharmacy to patients because the Company has not yet been able to reasonably estimate the third-party payor mix and resulting rebates based on its lack of sufficient historical data. Billings to the Company's distributor in advance of product shipment and delivery by the specialty pharmacy to patients are recorded as deferred revenue by the Company until such shipments to patients occur.

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

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

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 approval, the Company began capitalizing these costs and manufacturing overhead as commercial inventory. Upon launching PROCYSBI in mid-June 2013 in the U.S., 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; amortization of licensing approval milestone payments and licensing royalties payable to the University of California, San Diego ("UCSD").

Impairment of Goodwill and Intangible Assets

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

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

Fixed Assets

Fixed assets, which mainly consist of leasehold improvements, office furniture, manufacturing and 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.

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 condensed consolidated statements of operations. At the exercise date, the fair values of these warrants are re-measured and reclassified to equity.

Note Payable and Debt Issuance Costs

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

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 commercial drug manufacturing expenses prior to obtaining marketing approval.

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 income per share is calculated by dividing net income 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:

| | March 31, 2014 | 2013 |
|---|---|---|
| Warrants to purchase common stock | 334,764 | 3,962,772 |
| Options to purchase common stock | 9,584,383 | 7,885,393 |
| Total potentially dilutive securities | 9,919,147 | 11,848,165 |

Comprehensive Loss

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

Stock Option Plan

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

The Company recognizes expense associated with stock options issued to third parties, including consultants based upon the fair value of such awards on the date the options vest.

Income Taxes

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

The Company identifies uncertain tax positions and discloses any potential tax liability on their financial statements. The Company recognizes interest and/or penalties related to income tax matters as a component of income tax expense. As of March 31, 2014, 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 year presentation.

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.

Determining which category an asset or liability falls within the hierarchy requires significant judgment. The Company evaluates its hierarchy disclosures each quarter. Assets and liabilities measured at fair value on a recurring basis at March 31, 2014 and December 31, 2013 are summarized as follows:

| (In thousands) | Level 1 | Level 2 | Level 3 | Total |
|---|---|---|---|---|
| March 31, 2014: | | | | |
| Assets | | | | |
| Cash equivalents | $60,542 | $ 0 | $0 | $60,542 |
| Total | $60,542 | $ 0 | $0 | $60,542 |
| | | | | |
| Liabilities | | | | |
| Common stock warrants | $0 | $ 0 | $726 | $726 |
| Total | $0 | $ 0 | $726 | $726 |
| December 31, 2013: | | | | |
| Assets | | | | |
| Cash equivalents | $70,627 | $ 0 | $0 | $70,627 |
| Total | $70,627 | $ 0 | $0 | $70,627 |
| | | | | |
| Liabilities | | | | |
| Common stock warrants | $0 | $ 0 | $7,066 | $7,066 |
| Total | $0 | $ 0 | $7,066 | $7,066 |

Cash equivalents represent the fair value of the Company's investments in money market funds as of March 31, 2014 and December 31, 2013.

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 condensed consolidated statements of operations.

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

| (In thousands) | |
|---|---|
| Fair value as of December 31, 2013 | $7,066 |
| Change in fair value recognized in earnings | 1,163 |
| Exercises | (7,503) |
| Fair value as of March 31, 2014 | $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 the 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.

3. 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 the obligations, UCSD may terminate the license or otherwise cause the license to become non-exclusive.

In April 2013, the Company received FDA approval of 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 had 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, 2014 | December 31, 2013 |
|---|---|---|---|
| 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 | | (957 ) | (897 ) |
| Intangible assets, net | | $3,153 | $ 3,213 |

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, 2014 and year ended December 31, 2013, there was no intangible asset impairment recognized. During the three months ended March 31, 2014 and 2013, the Company amortized approximately $60 and $36, respectively, of intangible assets to research and development expense.

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

| Amortization period (In thousands) | Amortization expense |
|---|---|
| 2014 (remaining 9 months) | $ 178 |

| | |
|---|---|
| 2015 | 238 |
| 2016 | 238 |
| 2017 | 238 |
| 2018 | 238 |

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

4. INVENTORIES

Inventories consist of raw materials, work-in-process and finished goods related to the manufacture of PROCYSBI. Raw materials include the Company's active pharmaceutical ingredient ("API") for PROCYSBI. 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 and work-in-process that may be used for clinical trials, which are charged to research and development (R&D) expense when consumed.

Inventories are summarized as follows:

| (In thousands) | March 31, 2014 | December 31, 2013 |
|---|---|---|
| Raw materials | $3,136 | $ 2,469 |
| Work-in-process | 1,062 | 0 |
| Finished goods | 2,165 | 531 |
| Total inventories | $6,363 | $ 3,000 |

5. FIXED ASSETS

Fixed assets consisted of:

| (In thousands) | March 31, 2014 | December 31, 2013 | Estimated useful lives |
|---|---|---|---|
| Construction in progress | $103 | $ 0 | – |
| Office furniture | 605 | 605 | 7 years |
| Laboratory equipment | 1,227 | 1,132 | 5 years |
| Manufacturing equipment | 102 | 102 | 5 years |
| Computer hardware and software | 567 | 578 | 3 years |
| Capital lease equipment | 68 | 68 | Shorter of life of asset or lease term |
| Total at cost | 2,672 | 2,485 | |
| Less: accumulated depreciation | (792 ) | (675 ) | |
| Total fixed assets, net | $1,880 | $ 1,810 | |

Depreciation expense for the three months ended March 31, 2014 and 2013 was approximately $117 and $36, respectively. Accumulated depreciation on capital lease equipment was approximately $14 and $10 as of March 31, 2014 and December 31, 2013, respectively.

6. NOTE PAYABLE AND DEBT ISSUANCE COSTS

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 "HC Royalty Loan"). The Company received $23.4 million in net proceeds from the first tranche of the loan at closing in December 2012 and an additional $23.7 million in net proceeds in May 2013 from the second tranche upon FDA approval of PROCYSBI. The Company's 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 HC Royalty Loan, the Company granted a security interest to HC Royalty in substantially all of its assets, the assets of its domestic subsidiaries and a pledge of stock of certain of its domestic subsidiaries. The Company's failure to comply with the terms of the HC

Royalty Loan 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, could result in an event of default under the HC Royalty 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 could potentially take possession of, foreclose on, sell, assign or grant a license to use, the Company's pledged collateral and assign and transfer the pledged stock of certain of its subsidiaries.

The loan bears interest at an annual fixed rate of 10.75%, payable quarterly. The loan also contains a synthetic royalty component based on net product revenues, including PROCYSBI, in a calendar year, and such royalty is payable quarterly. With respect to the first $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bears a royalty rate of 6.25% of the first $25.0 million of product net revenues, 3.0% of product net revenues for such calendar year in excess of $25.0 million and up to $50.0 million, and 1.0% of product net revenues for such calendar year in excess of $50.0 million, payable quarterly. With respect to the second $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bears a royalty rate of 6.0% of the first $25.0 million of net revenues for such calendar year, 3.0% of product net revenues for such calendar year in excess of $25.0 million and 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 received marketing approval of PROCYSBI from the FDA on April 30, 2013 and commenced shipment of PROCYSBI during June 2013, and as a result, royalties became payable to HC Royalty based upon net revenues of PROCYSBI. Interest expense on the loan for the three months ended March 31, 2014 and 2013 was $2.8 million and $0.7 million, respectively.

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

Unamortized debt issuance costs totaled $2.6 million and $2.8 million as of March 31, 2014 and December 31, 2013, respectively. Amortization expense for the three months ended March 31, 2014 and 2013 was $0.1 million for both periods.

7. ACCRUED LIABILITIES

Accrued liabilities consisted of:

| (In thousands) | March 31, 2014 | December 31, 2013 |
|---|---|---|
| Clinical trials and research and development costs | $2,137 | $ 1,661 |
| Personnel-related costs | 2,668 | 4,443 |
| Rebates and other sales deductions | 2,921 | 2,325 |
| Royalty-based interest payable | 1,590 | 1,255 |
| License royalty payable | 564 | 564 |
| Manufacturing costs | 1,587 | 294 |
| Other | 1,778 | 2,225 |
| Total accrued liabilities | $13,245 | $ 12,767 |

8. CAPITAL STRUCTURE

Preferred Stock

At March 31, 2014, 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.

Common Stock

At March 31, 2014, 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, 2014 and December 31, 2013, there were 62,598,859 and 61,614,576 shares, respectively, of the Company's common stock issued and outstanding.

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 may, 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 may be raised to $100 million. Cowen is the sole sales agent for any sales made under the ATM for a 3.0% commission on gross proceeds. The common stock is sold at prevailing market prices at the time of the sale of common stock, and, as a result, prices will vary. During the three months ended March 31, 2014, there were no shares sold under the ATM. As of March 31, 2014, the Company had used approximately $53.8 million under the ATM.

Common Stock Warrants

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.

The table reflects the number of common stock warrants outstanding as of March 31, 2014:

| | Number of shares exercisable | Exercise price | Expiration date |
|---|---|---|---|
| Issued in connection with Encode merger | 233,309 | $2.87 | 12/13/2015 |
| TorreyPines warrants assumed in 2009 Merger | 3,502 | $157.08 | 9/26/2015 |
| Issued to registered direct investors in Dec. 2009 | 1 | $2.45 | 12/22/2014 |
| Issued to placement agent in Aug. 2010 | 97,952 | $3.075 | 8/12/2015 |
| Total warrants outstanding | 334,764 | $4.54 | * |

* Weighted-average exercise price

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

A Black-Scholes option-pricing model was used to obtain the fair value of the warrant liabilities using the following assumptions at March 31, 2014 and December 31, 2013:

| | December 2009 equity financing Series A | | August 2010 private placement Investors and placement agent | |
|---|---|---|---|---|
| | March 31, 2014 | December 31, 2013 | March 31, 2014 | December 31, 2013 |
| Fair value | $0 | $ 133 | $726 | $ 6,933 |
| Black-Scholes inputs: | | | | |
| Stock price | $10.00 | $ 13.02 | $10.00 | $ 13.02 |
| Exercise price | $2.45 | $ 2.45 | $3.075 | $ 3.075 |
| Risk free interest rate | 0.10 % | 0.13 % | 0.34 % | 0.33 % |
| Volatility | 95 % | 95 % | 95 % | 95 % |
| Expected term (years) | 0.75 | 1.0 | 1.5 | 1.75 |
| Dividend | 0 | 0 | 0 | 0 |

9. STOCK OPTION PLANS

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. During the three months ended March 31, 2014, the Company received approximately $1.2 million from the exercise of stock options in exchange for the issuance of 372,677 shares of the Company's common stock. As of March 31, 2014, there were 2,119,760 shares remaining available for issuance.

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

| | For the three months ended March 31, | |
|---|---|---|
| | 2014 | 2013 |
| Cost of goods sold | $40 | $0 |
| Research and development | 559 | 363 |
| Selling, general and administrative | 1,776 | 1,389 |
| Total stock-based compensation expense | $2,375 | $1,752 |

Stock-based compensation expense for consultants for the three months ended March 31, 2014 and 2013 was $0 and $4, respectively.

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

| | Option shares | Weighted-average exercise price |
|---|---|---|
| Outstanding at December 31, 2013 | 8,217,674 | $ 6.05 |
| Granted | 1,785,730 | 14.76 |
| Exercised | (372,677 ) | 3.11 |
| Canceled | (46,344 ) | 5.77 |
| Outstanding at March 31, 2014 | 9,584,383 | 7.70 |

10. 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 NAFLD clinical collaborations. The Company's contractual obligations did not change significantly during the three months ended March 31, 2014 compared to those disclosed as of December 31, 2013.

Contingencies

From time to time, the Company may have certain contingent liabilities that arise in the ordinary course of its business activities. The Company accrues a liability for such matters when it is probable a loss has been incurred and such loss can be reasonably estimated. At March 31, 2014, the Company has not recorded any material liabilities for contingencies.

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

You should read the following discussion in conjunction with our condensed consolidated financial statements as of March 31, 2014, and the notes to such 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. or Raptor Pharmaceuticals, Raptor European Products, LLC, RPTP European Holdings C.V., Raptor Pharmaceuticals Europe B.V., Raptor Pharmaceuticals France SAS and Raptor Pharmaceuticals Germany GmbH.

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, that plan for or anticipate the future. 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' actual operations, performance, development and results might differ materially from any forward-looking statement due to various known and unknown risks, uncertainties, assumptions and contingencies, including those described in the section titled "Risk Factors" in Part 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 any other reason.

Plan of Operation and Overview

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

the development stage.

Clinical Development Programs

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

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

Preclinical Product Candidates

Our preclinical programs, for which we may seek development partners in the future, include our cysteamine dioxygenase, or ADO, program and our HepTide™ program, for the potential treatment of hepatocellular carcinoma and other cancers susceptible to induced lysosomal storage.

Future Activities

Over the next fiscal year, our efforts will be focused on increasing sales of PROCYSBI in the U.S. and Germany; launching PROCYSBI in other countries in the EU; filing a New Drug Submission, or NDS, for cysteamine bitartrate delayed-release capsules with Health Canada in the second half of 2014; conducting a clinical trial to evaluate PROCYSBI in cystinosis patients that are cysteamine-naïve, as well as other supporting trials in underdeveloped markets; developing select global markets with significant numbers of known cystinosis patients; screening for undiagnosed and unidentified adult nephropathic cystinosis patients; supporting our regulatory pathways and/or clinical trials of RP103 for the potential treatment of HD, NAFLD, Leigh syndrome and mitochondrial disorders; preparing for potential clinical studies of RP103 in new therapeutic indications; supporting our novel preclinical programs; and identifying promising in-licensing candidates.

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

Results of Operations

Three months ended March 31, 2014 and 2013

Revenue

For the three months ended March 31, 2014, we recognized $12.1 million in PROCYSBI net product sales. The first U.S. sales of PROCYSBI commenced in June 2013 and the launch of PROCYSBI in Germany commenced in April 2014. There were no PROCYSBI product sales for the three months ended March 31, 2013.

Cost of Sales

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 for the next several quarters will reflect a lower average per unit cost of goods than will be recorded in the future. Cost of sales for the three months ended March 31, 2014 were $1.3 million and primarily included capitalized commercial product sold, amortization of licensing milestone payments, royalty fees payable to UCSD on our net product sales and other indirect costs such as distribution, labeling, shipping and supplies. We began capitalizing commercial inventory costs upon FDA approval of PROCYSBI on April 30, 2013.

Research and Development

Research and development expenses include medical, clinical, regulatory, quality, pharmacovigilance and research salaries and benefits; expenses associated with the manufacturing and testing of PROCYSBI inventory for our

commercial launch in the U.S. which were expensed prior to drug approval; 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 13% to $9.5 million for the three months ended March 31, 2014 from $8.4 million during the three months ended March 31, 2013. The increase of $1.1 million was primarily due to an increase in staffing and associated salaries and benefits for medical, clinical, quality and regulatory personnel, as well as increased spending for preclinical studies, clinical trials and non-commercial drug manufacturing expenses.

Major program expenses recorded as research and development expenses:

| | For the three months ended March 31, | |
|---|---|---|
| (In thousands) | 2014 | 2013 |
| RP103: | | |
| Cystinosis (pre-commercial and extension) | $3,661 | $4,179 |
| HD (clinical) | 504 | 212 |
| NAFLD (clinical) | 442 | 445 |
| Preclinical programs | 572 | 200 |
| Other programs | 653 | 254 |
| R & D personnel and other costs not allocated to programs | 3,715 | 3,122 |
| Total research and development expenses | $9,547 | $8,412 |

Selling, General and Administrative Expenses

Selling, general and administrative expenses primarily includes commercial expenses related to marketing and sales efforts 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; intellectual property, legal and audit fees, finance, executive and commercial operations salaries and benefits; and other administrative and facilities costs.

Selling, general and administrative expenses increased approximately 53% to $12.1 million for the three months ended March 31, 2014 from $7.9 million in the three months ended March 31, 2013. The $4.2 million increase was primarily due to $3.3 million for increased staffing and personnel-related expenses for the commercialization of PROCYSBI in the U.S., for the establishment of our EU commercial headquarters and build out of our German commercial team in anticipation of the recent launch of PROCYSBI in Germany and $0.9 million of increased spending for external services to support commercial operations.

Interest Expense

Interest expense for the three months ended March 31, 2014 and 2013 was $3.0 million and $0.7 million, respectively. The increase in interest expense was due primarily to the $50.0 million loan agreement that we entered into with HealthCare Royalty Partners II, L.P., or HC Royalty, in December 2012, of which net proceeds of $23.4 million and $23.7 million were received in December 2012 and May 2013, respectively. Interest expense for the three months ended March 31, 2014 also includes an interest expense royalty fee based on net sales for the quarter. We did not have product sales in the comparable period last year.

Adjustment to the Fair Value of Common Stock Warrants

The adjustment to the fair value of common stock warrants was loss of $1.2 million for the three months ended March 31, 2014 compared to a gain of $1.1 million for the three months ended March 31, 2013. The loss for the three months ended March 31, 2014 was due primarily to the increases in the price of our stock through the dates that warrants were exercised during the quarter. The gain for the three months ended March 31, 2013 was due primarily to a decrease in the volatility of our stock price. At March 31, 2014, the remaining warrants outstanding were 334,764 compared to 3,962,772 at March 31, 2013.

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., or Accredo, which is currently our only U.S. customer and ships directly to patients. Our commercial launch in Germany commenced in April 2014, with the Almac Group, Ltd. as our distributor in the EU. PROCYSBI is not available in U.S. retail pharmacies. Prior authorization of coverage by patients' commercial insurance plans, our patient assistance program, or PAP, or government payors is a prerequisite to the shipment of PROCYSBI to U.S. patients. Revenue is recognized once the product has been shipped by the specialty pharmacy to patients because we have not yet been able to reasonably estimate the third-party payor mix and resulting rebates based on our lack of sufficient historical data. Billings to our distributor in advance of product shipment and delivery by the specialty pharmacy to patients are recorded as deferred revenue by us until such shipments to patients occur.

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

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

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 approval, we began capitalizing these costs and manufacturing overhead as commercial inventory. Cost of sales includes the cost of inventory sold or reserved; manufacturing, manufacturing overhead and supply chain costs; product shipping and handling costs; amortization of licensing approval milestone payments and licensing royalties payable to UCSD.

Note Payable

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

Impairment of Goodwill and Intangible Assets

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

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

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

Common Stock Warrant Liabilities

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

We use the Black-Scholes option pricing model as our method of valuation for warrants that are subject to warrant liability accounting. The determination of the fair value as of the reporting date is affected by our stock price as well as assumptions regarding a number of highly complex and subjective variables. These variables include, but are not

limited to, expected stock price volatility over the term of the security and risk-free interest rate. In addition, the Black-Scholes option pricing model requires the input of an expected life for the securities for which we have estimated based upon the stage of our development. The fair value of the warrant liability is revalued each balance sheet date utilizing Black-Scholes valuation model computations with the decrease or increase in fair value being reported in the statement of operations and comprehensive loss as other income or expense, respectively. The primary factors affecting the fair value of the warrant liability are our stock price and volatility.

We reported a net loss of $14.9 million for the three months ended March 31, 2014. If our March 31, 2014 closing stock price had been 10% lower, our net loss would have been approximately $0.1 million lower. If our March 31, 2014 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, 2014 would not have had a material effect on our net loss due to the lower number of warrants that remain outstanding at March 31, 2014.

Income Taxes

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

We identify uncertain tax positions and record or disclose any resulting potential tax liability based upon whether the position is more likely than not sustainable upon examination. We consider proposed assessments by tax authorities, changes in facts and circumstances, issuance of new regulations or new case law and negotiations between tax authorities of different countries concerning our transfer prices or intellectual property transfers. As of March 31, 2014, we have 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, 2014, we had approximately $68.1 million in cash and cash equivalents, approximately $21.3 million in current liabilities (of which approximately $0.7 million represented the common stock warrant liability, which is expected to be settled in shares) and approximately $64.2 million of net working capital.

We estimate that without regard to any other future sources of funds, our cash and cash equivalents of approximately $68.1 million as of March 31, 2014 will be sufficient to meet our projected operational requirements and obligations at least through the first half of 2015.

Under the terms of the HC Royalty loan agreement executed on December 20, 2012, we received $23.4 million in net proceeds from the first tranche of the loan at closing in December 2012. We received an additional $23.7 million in net proceeds in May 2013 from the second tranche upon FDA approval of PROCYSBI. No additional funds can be borrowed under the loan agreement without HCR's consent. The loan matures on March 31, 2020, bears interest at an annual fixed rate of 10.75% and has a synthetic royalty, tiered down, based on a percentage of net product sales. The loan is interest-only until May 2015. The proceeds from the loans are being used primarily to fund the commercialization of PROCYSBI for the management of cystinosis, advance our development programs and for general corporate purposes.

On April 30, 2012, we entered into a Sales Agreement with Cowen and Company, or Cowen, to sell shares of our common stock, with aggregate gross sales proceeds of up to $40 million, from time to time through an "at the market" , or ATM, equity offering program under which Cowen acts as sales agent. We pay a 3% commission to Cowen on any sales pursuant to this Sales Agreement. On July 3, 2013, we amended and restated the Sales Agreement to increase the aggregate gross sales proceeds that may be raised to $100 million. Cumulatively through March 31, 2014, we sold 7,599,474 shares under the ATM offerings at a weighted-average selling price of $7.08 per share for net proceeds of approximately $52.1 million. As of March 31, 2014, we had used approximately $53.8 million under the ATM.

Future Funding Requirements

We will need to raise additional capital either through the sale of equity or debt securities (including convertible debt securities) to fund our operations and to, among other activities, 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 U.S., including patient uptake;
- the ongoing costs of establishing the sales and marketing capabilities in the EU necessary to successfully launch PROCYSBI in the EU;
- our ability to negotiate reimbursement and pricing of PROCYSBI in various countries in the EU;
- the successful launch of PROCYSBI in other countries in the EU;
- the cost of our manufacturing-related activities in support of PROCYSBI and RP103;
- the cost of activities related to the regulatory submission of 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-EU 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 Huntington's disease; evaluating RP103 as a potential treatment for NAFLD; 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 indication using RP103;
- the cost of evaluating and potentially acquiring or in-licensing new drug compound(s) for potential clinical development;
- the cost of business development activities to identify, test and potentially license or acquire new therapeutic drug candidates; and
- the cost of filing, prosecuting and enforcing patent claims.

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 NAFLD 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, 2014 compared to those discussed in our Annual Report on Form 10-K for the year ended December 31, 2013, filed with the SEC on March 17, 2014.

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 quarter ended March 31, 2014 have not materially changed from those discussed in our Annual Report on Form 10-K for the year ended December 31, 2013, filed with the SEC on March 17, 2014.

ITEM 4. CONTROLS AND PROCEDURES

Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and our 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 that evaluation, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures as of such date are effective, at the reasonable assurance level, in ensuring that information required to be disclosed by us in the reports that we file or submit under the Securities Exchange Act of 1934, as amended, or the Exchange Act, is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. Our disclosure controls and procedures were designed to provide reasonable assurance of achieving our control objectives.

Changes in Internal Control over Financial Reporting

During the three months ended March 31, 2014, there were no changes in our internal control over financial reporting that have materially affected or are reasonably likely to have a material impact on our internal control over financial reporting.

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 consider carefully the specific risks detailed in this "Risk Factors" section, together with all of the other information contained in this Quarterly Report on Form 10-Q. 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

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

PROCYSBI is our only product currently approved for marketing and, as a result, our operating results are substantially dependent on the commercial success of PROCYSBI, for which we commenced marketing in the U.S. in June 2013 and in Germany in April 2014. In the U.S., we are permitted to market PROCYSBI only for the management of nephropathic cystinosis in adults and children six years and older. In September 2013, we received marketing authorization from the EC, which allows us to commercialize PROCYSBI in the 28 Member States of the EU plus Norway, Liechtenstein and Iceland (which are not EU Member States but are part of the EFTA), for the treatment of proven nephropathic cystinosis; however, we only recently commenced our commercial launch of PROCYSBI in Germany and have not yet launched in any other country in the EU. We believe that the trading price of our common stock will be substantially affected by our results of operations and, in particular, net product sales of PROCYSBI. We do not have prior experience in commercializing therapeutics. If PROCYSBI sales do not meet expectations, our stock price may not increase or could significantly decrease. The successful commercialization of PROCYSBI will depend on several factors, including:

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

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

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

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

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

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

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

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

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

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

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

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

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

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

Using external contract manufacturing organizations, or CMOs, we currently manufacture commercial and clinical quantities of PROCYSBI and RP103 for the indications under development. We rely on single manufacturing sources for our cysteamine active pharmaceutical ingredient, or API, and finished products. Our ability to obtain sufficient quantities of PROCYSBI and RP103 is constrained by limited supplies of raw materials and capacity and output of these manufacturers. Furthermore, any reduction or interruption in our supply of API from the single source supplier and of finished goods from our CMO, and efforts to identify and qualify alternative sources of API supply, could result in significant additional operating costs, interruptions in product supply and delays in sales of PROCYSBI and in developing RP103 for HD, NAFLD and Leigh's syndrome. In addition, supply arrangements from alternative sources may not be available on acceptable economic terms, if at all.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

In 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (together, the "Health Care Reform Law"), a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The Health Care Reform Law, among other things, revised the definition of AMP for reporting purposes, which could increase the amount of Medicaid drug rebates to states and extended the rebate program to beneficiaries enrolled in Medicaid managed care organizations. The Health Care Reform Law also imposed a significant annual fee on companies that manufacture or import branded prescription drug products and established an annual non-deductible fee on entities that sell branded prescription drugs or biologics to specified government programs in the U.S. The Health Care Reform Law also expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance and included a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or "donut hole." The Health Care Reform Law includes a provision to increase the Medicaid rebate for line extensions or reformulated drugs, which depending on how this provision is implemented could substantially increase our Medicaid rebate rate (in effect limiting reimbursement for these patients). These and other new provisions are likely to continue the pressure on pharmaceutical pricing, may require us to modify our business practices with healthcare practitioners, and may also increase our regulatory burdens and operating costs. See the risk factor titled "Failure to comply with healthcare regulations may subject us to substantial penalties."

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

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

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

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

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in exchange for or to induce either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs, such as the Medicare and Medicaid programs;
- federal false claims laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid or other federal third-party payors that are false or fraudulent;
- federal criminal laws that prohibit executing a scheme to defraud any federal healthcare benefit program or making false statements relating to healthcare matters;
- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information;
- the federal Physician Payment Sunshine Act, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments or other "transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, and applicable manufacturers and group purchasing organizations to report annually to CMS ownership and investment interests held by physicians (as defined above) and their immediate family members and payments or other "transfers of value" to such physician owners and their immediate family members. Manufacturers were required to begin data collection on August 1, 2013 and to report aggregate data to the government by March 31, 2014 with more detailed reports due by August 1, 2014;
- in the EU, in various Member States, including France, the UK, the Netherlands, Italy, or Spain, the legislator or self-regulatory industry bodies have adopted rules requiring the notification and/or publication of certain transfers of value from pharmaceutical companies to health care professionals. For example, France has recently adopted legislation (Law No. 2011-2012, or the "French Sunshine Act," and Decree no. 2013-414 which implements it) requiring pharmaceutical companies to disclose and publish agreements with or transfers of value to health care professionals; and
- analogous state and foreign law equivalents of each of the above federal laws, including, but not limited to anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers.

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

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

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available under such laws, it is possible that some of our business activities, including our relationships with physicians and other health care providers, some of whom recommend, purchase and/or prescribe our products, could be subject to

challenge under one or more of such laws. While these activities are structured to comply with all applicable laws, if our operations are found to be in violation of any of the laws described above or any other laws and regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, the exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to market PROCYSBI, RP103 and other future drug candidates and adversely impact our financial results. See also the risk factor titled "If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the U.S., we could be subject to additional reimbursement requirements, penalties, sanctions and fines which could have a material adverse effect on our business, financial condition, results of operations and future business prospects."

As we expand our development and commercialization activities outside of the U.S., we will be subject to an increased risk of inadvertently conducting activities in a manner that violates the U.S. Foreign Corrupt Practices Act, or FCPA, and similar laws. If that occurs, we may be subject to civil or criminal penalties which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We are subject to the U.S. 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. We are also subject to the UK Anti-Bribery Act, which prohibits both domestic and international bribery, as well as bribery across both public and private sectors.

In the course of establishing and expanding our commercial operations and seeking regulatory approvals outside of the U.S., 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, 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 substantial fines and penalties and could materially harm our business. Furthermore, any finding of a violation under one country's laws may increase the likelihood that we will be prosecuted and be found to have violated another country's laws. If our business practices outside the U.S. are found to be in violation of the FCPA, UK Bribery Act or other similar law, we may be subject to significant civil and criminal penalties which could have a material adverse effect on our business, financial condition, results of operations, liquidity and growth prospects.

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

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

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

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

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

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

PROCYSBI and our clinical development of RP103 target diseases with small patient populations, including cystinosis and HD, respectively. A key component of the successful commercialization of a drug product for these indications includes identification of patients and a targeted prescriber base for the drug product. Due to small patient populations, we believe that we would need to have significant market penetration to achieve meaningful revenues and identifying patients and targeting the prescriber base are key to achieving significant market penetration. In addition, the per-patient prices at which we sell PROCYSBI (currently estimated at an average of $265,000 per year after rebates, discounts, distribution fees and adjusted for patient compliance in the U.S.) and RP103 for these indications will need to be relatively high in order for us to generate an appropriate return for the investment in these product development programs and achieve meaningful gross margins. There can be no assurance that we will be successful in achieving a sufficient degree of market penetration and/or obtaining or maintaining high per-patient prices for PROCYSBI and RP103 for diseases with small patient populations. Further, even if we obtain significant market share for PROCYSBI and RP103, if approved, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share. Additionally, patients who discontinue therapy or do not fill prescriptions are not easily replaced by new patients, given the limited patient population.

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

As part of our business strategy, we intend to develop RP103 for additional indications and other drugs that may be eligible for FDA and EMA orphan drug designation. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, defined as a patient population of less than 200,000 in the U.S., or a patient population greater than 200,000 in the U.S. where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the U.S., orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated condition for a period of seven years, with an additional six months if for a pediatric indication. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective, a subsequent product is deemed clinically superior, or if the manufacturer is unable to deliver sufficient quantity of the drug.

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

Because the extent and scope of patent protection for some of our drug products may be particularly limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible drugs, we plan to rely on the orphan exclusivity period to maintain a competitive position. However, if we do not obtain orphan drug exclusivity for RP103 for the potential treatment of HD or other potential indications, or our future

relevant drug products do not have strong patent protection, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced. Also, without strong patent protection, competitors may sell a generic version upon the expiration of orphan exclusivity, if our patent position is not upheld.

Even though we have been granted orphan drug designation in the U.S. prior to the approval of RP103 for the potential treatment of HD, and even if we obtain orphan drug designation for our future drug product candidates, we may not fulfill the criteria for exclusivity or we may not be the first to obtain marketing approval for any orphan indication. Further, even if we obtain orphan drug exclusivity for a particular product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve a drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. The FDA can discontinue orphan drug exclusivity after it has been granted if the orphan drug cannot be manufactured in sufficient quantities to meet demand. Positive clinical trial results in any of our RP103 programs increase the risk that immediate-release cysteamine bitartrate may be used off-label in those indications in certain geographic areas due to immediate-release cysteamine bitartrate's lower cost and our 505(b)(2) filing status.

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

Under the Prescription Drug User Fee Act, the FDA has a goal of responding to NDAs within 10 months of submission the filing date for standard review, but this timeframe is also often extended. In the future, we may seek approval of our drug candidates under programs designed to accelerate the FDA's review and approval of NDAs. For example, a sponsor may seek FDA designation of a drug candidate as a "fast track product." Fast track products are those products intended for the treatment of a serious or life-threatening disease or condition and which demonstrate the potential to address unmet medical needs for such disease or condition. If fast track designation is obtained, the FDA may initiate review of sections of an NDA before the application is complete. This "rolling review" is available if the applicant provides and the FDA approves a schedule for the remaining information. In some cases, a product may be approved on the basis of either a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Approvals of this kind typically include requirements for appropriate post-approval Phase 4 clinical trials to validate the surrogate endpoint or otherwise confirm the effect of the clinical endpoint. In addition, the Food and Drug Administration Safety and Innovation Act, or FDASIA, which was enacted and signed into law in 2012, established a new category of drugs referred to as "breakthrough therapies," which are defined as drugs intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. In the future, we may request breakthrough designation or fast track designation from the FDA for our other drug product candidates, but we cannot assure that we will obtain such designations. Moreover, even if we obtain breakthrough designation or fast track designation from the FDA, the designations do not guarantee FDA approval of our NDA, that the development program or review timeline will ultimately be shorter than if we had not obtained the designations, or that the FDA will not request additional information, including requesting additional clinical studies (although potentially a post-marketing requirement), during its review. Any request for additional information or clinical data could delay the FDA's timely review of our NDA.

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

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

- the potential strain on our financial and managerial controls and reporting systems and procedures;
- potential miscommunication between U.S. personnel and European personnel due to cultural and language differences;
- the small size of our company and our intention to grow at a consistent but measured pace;
- ability to operate within diverse individual country regulatory and statutory laws; and
- the costs of maintaining EU presence, in-country legal entities and related tax structures.

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

Sales of our products outside of the U.S. are subject to foreign regulatory requirements governing clinical trials, manufacturing and marketing approvals. Even if the FDA and EC grant marketing approval for a product candidate, comparable regulatory authorities of foreign countries must also approve the manufacturing and marketing of our product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the U.S., including additional preclinical

studies or clinical trials or manufacturing and control requirements. In many countries outside the U.S., a product candidate must be approved for reimbursement before it can be approved for sale in that country. In many cases, the price that we propose to charge for our products is also subject to approval by individual countries before we can launch our product candidates in those countries. Obtaining foreign regulatory approvals, complying with foreign regulatory requirements and gaining approved pricing and reimbursement could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries and regulatory approval in one country does not ensure approval in any other country, while a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome and results of earlier studies and trials may not be predictive of future trial results. If we fail to demonstrate safety or efficacy in our preclinical studies or clinical trials or keep to the terms of a product development program, our future business prospects for these drug product candidates will be materially adversely affected.

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

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

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

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

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

- slower than expected rates of patient recruitment and enrollment;

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

In addition, many of our clinical trials involve small patient populations. Because of the small sample size, the results of these early clinical trials may not be indicative of future results. Any delay in our preclinical or clinical programs or the failure to demonstrate safety or efficacy in our clinical trials would have a material adverse effect on our future business prospects, financial condition and results of operations.

We may be subject to product liability claims.

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

We may not be able to avoid significant liability if any product liability claim is brought against us. Although we currently carry product liability insurance, it may not be sufficient to cover future claims. If a successful product liability claim is brought against us and the amount of liability exceeds our insurance coverage, we may incur substantial charges that would adversely affect our business, financial condition and results of operations.

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

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

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

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

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

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

- 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 pivotal to establishing products using our technologies as a standard of care for their approved indications. Although we have various medical and scientific advisors and research collaborations, there is no assurance that our advisors and our research collaborators will continue to work with us or that we will be able to attract additional research partners. If we are not able to maintain existing or establish new clinical and scientific relationships to assist in our commercialization and research and development, we may not be able to successfully develop PROCYSBI, RP103 or our other drug product candidates.

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

We face burdens relating to increased corporate governance and financial reporting standards. Legislation or regulations, such as the Physician Payment Sunshine Act, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and the Dodd-Frank Wall Street Reform and Consumer Protection Act, as well as other rules implemented by the FDA, the SEC and NASDAQ, follow stricter corporate governance and financial reporting standards and have led to an increase in the costs of compliance, including substantial increases in consulting, auditing and legal fees. Our management and other personnel will need to devote a substantial amount of time to these requirements. New rules could make it more difficult or more costly for us to obtain certain types of insurance, including directors' and officers' liability insurance, and we may be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, on our board committees or as executive officers. Failure to comply with these new laws and regulations may impact our financial condition and could materially harm our business.

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

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

Continued commercial sales of PROCYSBI in the U.S., the EU commercial launch of PROCYSBI, expansion of our commercial operations into other markets, the continuation of our clinical-stage programs and our current plans to in-license and acquire additional clinical-stage product candidates will require us to retain existing and add required new qualified and experienced personnel in all functional areas over the next several years. Also, if our preclinical pipeline diversifies through internal discoveries, or the acquisition or in-licensing of new molecules, we will need to hire additional scientists to supplement our existing scientific expertise over the next several years.

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

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

In December 2012, we entered into a loan agreement with HC Royalty as lender, under which we agreed to borrow $50.0 million in two $25.0 million tranches, or the HC Royalty loan agreement. We drew down the first tranche in the amount of $25.0 million in December 2012 upon signing the HC Royalty loan agreement and we drew down the second tranche of $25.0 million in May 2013 as a result of our achievement of the milestone of U.S. approval of PROCYSBI. The HC Royalty loan agreement includes a number of affirmative and negative covenants, including the use of commercially reasonable efforts to exploit PROCYSBI and RP103 in specific markets and compliance with laws, as well as restrictions on mergers and sales of assets, incurrence of liens, incurrence of indebtedness and transactions with affiliates and other requirements. To secure the performance of our obligations under the HC Royalty loan agreement, we granted a security interest to HC Royalty in substantially all of our assets, the assets of our domestic subsidiaries and a pledge of stock of certain of our domestic subsidiaries. Our failure to comply with the

terms of the HC Royalty loan agreement and related documents, the occurrence of a change of control of our Company or the occurrence of an uncured material adverse effect on our Company, or the occurrence of certain other specified events, could result in an event of default under the HC Royalty loan agreement that, if not cured or waived, could result in the acceleration of the payment of all of our indebtedness to HC Royalty and interest thereon. Under the terms of the security agreement, in an event of default, the lender could potentially take possession of, foreclose on, sell, assign or grant a license to use our pledged collateral and assign and transfer the pledged stock of certain of our subsidiaries. A default or material adverse effect or change of control would also trigger a prepayment penalty which would require us to pay a substantially higher amount due than the current balance of our loan.

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

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

Our business could be adversely affected by macroeconomic conditions.

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

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

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

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

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

As we continue with the commercial launch of PROCYSBI in Germany and initiate launches in other countries in the EU and in other countries outside the U.S., a portion of our business will be conducted in currencies other than our reporting currency, the U.S. dollar. We will recognize foreign currency gains or losses arising from our operations in the period in which we incur those gains or losses. As a result, currency fluctuations between the U.S. dollar and the currencies in which we do business will likely cause foreign currency translation gains and losses in the future. Because of the number of currencies that may be involved, the variability of currency exposures and the potential volatility of currency exchange rates, we may suffer significant foreign currency translation and transaction losses in the future due to the effect of exchange rate fluctuations.

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

Our success is dependent in part upon the availability of our senior executive officers, including Christopher M. Starr, Ph.D., Chief Executive Officer; Julie Anne Smith, Chief Operating Officer; Georgia Erbez, Chief Financial Officer and Ted Daley, Chief Business Officer. The loss or unavailability to us of any of these individuals or key research and development personnel, and particularly if lost to competitors, could have a material adverse effect on our business, prospects, financial condition and results of operations. We do not have key-man insurance on any of our employees.

There is no assurance that we will be able to retain key employees or consultants. There is intense competition for qualified scientists and managerial personnel from numerous pharmaceutical and biotechnology companies, as well as from academic and government organizations, research institutions and other entities. If key employees terminate

their employment, or if insufficient numbers of qualified employees are retained, or are not available via recruitment, to maintain effective operations, our development activities might be adversely affected, management's attention might be diverted from managing our operations to hiring suitable replacements and our business might suffer. In addition, we might not be able to locate suitable replacements for any key employees that terminate their employment with us and we may not be able to offer employment to potential replacements on reasonable terms, which could negatively impact our product candidate development timelines and may adversely affect our future revenues and financial condition.

In addition to our employees, we rely and will continue to rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development strategy. All of our consultants and advisors will be employed by other employers or be self-employed, and will have commitments to or consulting or advisory contracts with other entities that may limit their availability to us.

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

For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory, market launch and commercialization goals, which we sometimes refer to as milestones. These milestones include the completion of reimbursement and pricing negotiations and commercial launches in various EU countries and other territories, commencement or completion of scientific studies and clinical trials, and the submission of regulatory filings.

From time to time, we may publicly announce the estimated timing of some of these milestones. All of these milestones will be based on a variety of assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in many cases for reasons beyond our control. For example, clinical trials may be delayed due to factors such as IRB approvals, qualification of clinical sites, scheduling conflicts with participating clinicians and clinical institutions and the rate of patient enrollment. In most circumstances, we rely on academic institutions, major medical institutions, governmental research organizations (U.S. or internationally based), clinical research organizations or CMOs to conduct, supervise or monitor some or all aspects of clinical trials involving our product candidates. We will have limited control over the timing and other aspects of these clinical trials. Furthermore, our ability to launch commercial sales of PROCYSBI in various countries in the EU is subject to the timely completion of reimbursement and pricing negotiations with various governmental entities in the EU, which process can be lengthy and uncertain. See also the risk factors titled "The amount of our product sales of PROCYSBI in the EU is dependent upon the pricing and reimbursement guidelines adopted in each of the various countries in the EU, which levels may be below our current expectations" and "Our reliance on third parties may result in delays in completing, or a failure to complete, preclinical testing, clinical trials or regulatory marketing submissions."

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

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

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

Risks Related to Intellectual Property and Competition

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

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

The patent positions of biopharmaceutical products are complex and uncertain.

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

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

Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us, or file patent applications before we do. Competitors may also claim that we are infringing on their patents and therefore cannot practice our technology as claimed under our patents, if issued.

- Competitors may also contest our patents, if issued, by showing the patent examiner that the invention was not original, was not novel or was obvious. In litigation, a competitor could claim that our patents, if issued, are not valid for a number of reasons. If a court agrees, we would lose that patent. As a Company, we have no meaningful experience with competitors interfering with our patents or patent applications;

- Enforcing patents is expensive and may absorb significant management time. Management would spend less time and resources on developing drug product candidates. The processes of defending patents and related intellectual property could increase our operating expenses and delay product programs; and

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

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

- Defending a lawsuit takes significant time is typically very expensive;

- If a court decides that our drug product candidate infringes on the competitor's patent, we may have to pay substantial damages for past infringement;

- A court may prohibit us from selling or licensing the drug product candidate unless the patent holder licenses the patent to us. The patent holder is not required to grant us a license. If a license is available, we may have to pay substantial royalties or grant cross licenses to our patents; and

- Redesigning our drug product candidates so we do not infringe may not be possible or practical and could require substantial funds and time.

Our trade secrets may not be adequately protected. While we use reasonable efforts to protect our trade secrets, our employees or consultants may unintentionally or willfully disclose our information to competitors. Enforcing a claim that someone else illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the U.S. are sometimes less willing to protect trade secrets. Our competitors may independently develop equivalent knowledge, methods and know-how.

We may also support and collaborate in research conducted by government organizations, hospitals, universities or other educational institutions. These research partners may be unwilling to grant us any exclusive rights to technology or products derived from these collaborations prior to entering into the relationship. If we do not obtain required licenses or rights, we could encounter delays in our product development efforts while we attempt to design around other patents or even be prohibited from developing, manufacturing or selling drug product candidates requiring these licenses. There is also a risk that disputes may arise as to the rights to technology or drug product candidates developed in collaboration with other parties.

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

We have acquired and licensed certain proprietary technologies and plan to further license and acquire various patents and proprietary technologies owned by other parties. The agreements in place are critical to our product development programs. These agreements may be terminated, and all rights to the technologies and product candidates will be lost, if we fail to perform our obligations under these agreements and licenses in accordance with their terms including, but not limited to, our ability to fund all payments due under such agreements. Our inability to continue to maintain these technologies could adversely affect our business, financial condition and results of operations. In addition, our

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

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

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

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

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

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

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

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

Risks Related to Our Financial Position and Capital Requirements

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

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

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

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

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

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

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

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

As of March 31, 2014, we had an accumulated deficit of approximately $220.2 million. We will need to raise additional capital and/or generate significant revenue at profitable levels to fund our development and commercialization programs in accordance with our plans.

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

While we believe that based upon our projected PROCYSBI sales and planned operations, our cash and cash equivalents of $68.1 million as of March 31, 2014 will be sufficient to meet our projected operational requirements and obligations through at least through the first half of 2015, in the future, we may need to sell equity or debt securities to raise additional funds to support, among other things, our development and commercialization programs. The sale of additional equity securities or convertible debt securities will result in additional dilution to our stockholders. Additional financing may not be available on a timely basis, in amounts or on terms satisfactory to us, or at all. We may be unable to raise additional capital due to a variety of factors, including our financial condition, the status of our research and development programs, the status of regulatory reviews for marketing approvals, the status of our commercialization activities, sales of PROCYSBI in the U.S., the execution of our launch of PROCYSBI in Europe and the general condition of the financial markets. If we fail to raise additional financing when needed, we may have to delay or terminate some or all of our research and development programs, scale back our operations and/or reduce our commercial expenses for PROCYSBI. If such actions are required, our financial condition and operating results will be adversely affected and our future value may be significantly reduced.

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

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

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

Risks Related to Our Common Stock

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

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

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

- the inability to sell a product at the price we expect; or
- the inability to supply enough product to meet demand.

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

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

Our common stock is quoted on the NASDAQ Global Market. The trading price of our common stock has been and may continue to be volatile. Our operating performance, both financial and in the development of approved products, affects and will continue to significantly affect the market price of our common stock. We face a number of risks including those described in this Risk Factors section, which may negatively impact the price of our common stock. The market price of our common stock also may be adversely impacted by broad market and industry fluctuations including general economic and technology trends, regardless of our operating performance. The NASDAQ Global Market has, from time to time, experienced extreme price and trading volume fluctuations, and the market prices of securities of pharmaceutical, biotechnology and other life sciences companies in a comparable stage to us have historically been particularly volatile and trading volume in such securities has often been relatively small. Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. The stock market also has periods during which industry segments, such as biotechnology, are in volatile swings of greater or lesser favor as investments. These swings may affect in particular the stock prices of companies such as ours that do not have conventional measures of financial and business health such as sales, earnings, profitability and related measures.

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

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

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

We have entered into an Amended and Restated Sales Agreement with Cowen and Company, which, if utilized further, will create substantial dilution for our existing stockholders. The original Sales Agreement provided for at-the-market sales of our common stock with aggregate gross proceeds of up to $40.0 million. On July 3, 2013, we entered into an Amended and Restated Sales Agreement to increase the aggregate gross sales proceeds that may be raised pursuant to the agreement to $100.0 million. As of March 31, 2014, we had used approximately $53.8 million under the ATM.

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

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

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

common stock will appreciate in value or even maintain its current price. Investors seeking dividend income should not invest in our common stock.

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

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

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law as currently in effect may make a change in control of our Company more difficult, even if a change in control may be beneficial to the stockholders. Our board of directors has the authority to issue up to 15.0 million shares of preferred stock, none of which are issued or outstanding. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third-party to acquire a majority of our outstanding voting stock. Our certificate of incorporation contains provisions that may enable our management to resist an unwelcome takeover attempt by a third party, including: a prohibition on actions by written consent of our stockholders; the fact that stockholder meetings must be called by our board of directors; and provisions requiring stockholders to provide advance notice of proposals. Delaware law also prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, the board of directors approves the transaction. Our board of directors may use these provisions to prevent changes in the management and control of our Company. Also, under applicable Delaware law, our board of directors may adopt additional anti-takeover measures in the future.

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

## ITEM 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.

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 8, 2014 By: /s/ Christopher M. Starr
Christopher M. Starr, Ph.D.
Chief Executive Officer and Director
(Principal Executive Officer)

Date: May 8, 2014 By: /s/ Georgia Erbez
Georgia Erbez
Chief Financial Officer, Secretary and Treasurer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit Index

| Exhibit Number | Exhibit Description | Incorporated by Reference | | | Filed Herewith |
|---|---|---|---|---|---|
| | | Form | Date | Number | |
| 2.1 | Agreement and Plan of Merger and Reorganization, dated June 7, 2006, among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc. | | | | |
| 2.2 | Amendment No. 1 to Agreement and Plan of Merger and Reorganization, dated August 25, 2006, among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc. | | | | |
| 2.3 | Agreement and Plan of Merger and Reorganization, dated July 27, 2009, among ECP Acquisition, Inc., Raptor Pharmaceuticals Corp. and TorreyPines Therapeutics, Inc. | | | | |
| 3.1 | Certificate of Incorporation of Registrant | 8-K | 10/10/2006 | 3.1 | |
| 3.2 | Amended and Restated Bylaws of Registrant | 8-K | 2/26/2014 | 3.1 | |
| 3.3 | Certificate of Amendment filed with the Secretary of State of the State of Nevada effecting an 8-for-1 reverse stock of Registrant's common stock and changing the name of Registrant from Axonyx Inc. to TorreyPines Therapeutics, Inc. | 8-K | 10/10/2006 | 3.3 | |
| 3.4 | Articles of Conversion filed with the Secretary of State of the State of Nevada changing the state of incorporation of Registrant | 8-K | 10/10/2006 | 3.4 | |
| 3.5 | Certificate of Conversion filed with the Secretary of State of the State of Delaware | 8-K | 10/10/2006 | 3.5 | |
| 3.6 | Certificate of Amendment of Certificate of Incorporation of Registrant | 8-K | 10/5/2009 | 3.1 | |
| 3.7 | Certificate of Merger of ECP Acquisition, Inc. with and into Registrant | 8-K | 10/5/2009 | 3.2 | |
| 4.1 | Specimen common stock certificate of the Registrant | 8-K/A | 10/7/2009 | 4.7 | |
| 4.2(a) | Rights Agreement, dated as of May 13, 2005, between Registrant and The Nevada Agency and Trust Company, as Rights Agent | 8-K | 5/16/2005 | 99.2 | |
| 4.2(b) | Amendment to Rights Agreement, dated as of June 7, 2006, between Registrant and The Nevada Agency and Trust Company, as Rights Agent | 8-K | 6/12/2006 | 4.1 | |
| 4.2(c) | Amendment to Rights Agreement, dated as of October 3, 2006, between Registrant and The Nevada Agency and Trust Company, as Rights Agent | 10-K | 3/29/2007 | 4.19 | |
| 4.2(d) | Rights Agreement Amendment, dated as of July 27, 2009, to the Rights Agreement dated May 13, 2005 between Registrant and American Stock Transfer and Trust Company (replacing The Nevada Agency and Trust Company) | 8-K | 7/28/2009 | 2.3 | |
| 4.2(e) | Amendment to Rights Agreement, dated August 6, 2010, by and between Registrant and American Stock Transfer & Trust Company, LLC | 8-K | 8/10/2010 | 4.2 | |
| 4.3 | Form of Warrant, dated September 27, 2005, issued to Oxford Financial and Silicon Valley Bank. | 10-K | 3/29/2007 | 4.16 | |
| 4.4* | Warrant, dated December 14, 2007, issued to Flower Ventures, LLC. | 10-QSB** | 4/15/2008 | 4.1 | |

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|---|---|---|---|---|
| 4.5* | Warrant Agreement Amendment, dated December 17, 2009, between Flower Ventures, LLC and the Registrant. | 10-Q | 4/9/2010 | 4.15 |

| | | |
|---|---|---|
| 31.1 | Certification of Christopher M. Starr, Ph.D., Chief Executive Officer | X |
| 31.2 | Certification of Georgia Erbez, Chief Financial Officer, Secretary and Treasurer | X |
| 32.1 | Certification of Christopher M. Starr, Ph.D., Chief Executive Officer, and Georgia Erbez, 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, 2014, formatted in Extensible Business Reporting Language (XBRL): (i) the Condensed Consolidated Balance Sheets; (ii) the Condensed Consolidated Statements of Operations; (iii) the Condensed Consolidated Statements of Comprehensive Loss; (iv) the Condensed Consolidated Statements of Cash Flows; and (v) related notes, tagged as blocks of text. | X |

* The Raptor Pharmaceuticals Corp. warrants set forth in Exhibits 4.4 - 4.5 have been converted into warrants of the Registrant, and the exercise price of such warrants and number of shares of common stock issuable thereunder have been converted as described in Item 1.01 (under the section titled, "Background") of the Registrant's Current Report on Form 8-K, filed on October 5, 2009.

** Incorporated by reference from the indicated filing of Raptor Pharmaceuticals Corp. rather than that of the Registrant

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