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

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

**Risks Related to Our Common Stock**

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

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

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

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

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

Our common stock is quoted on the NASDAQ Global Market. The trading price of our common stock has been and may continue to be volatile. Our operating performance, both financial and in the development of approved products, affects and will continue to significantly affect the market price of our common stock. We face a number of risks including those described in this Risk Factors section, which may negatively impact the price of our common stock.

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

These broad market fluctuations, during which our stage of company and our industry may experience a stronger degree of market sensitivity, will adversely affect the trading price of our common stock. In the past, following periods of volatility in the market resulting in substantial price declines of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation can result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation. A substantial number of shares of our common stock are eligible for future sale in the public market, and the issuance or sale of equity, convertible or exchangeable securities in the market, or the perception of such future sales or issuances, could lead to a decline in the trading price of our common stock.

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

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

In connection with other collaborations, joint ventures, license agreements or future financings that we may enter into in the future, we may issue additional shares of common stock or other equity securities, and the value of the securities issued may be substantial and create additional dilution to our existing and future common stockholders. Because we do not intend to pay any cash dividends on our common stock, investors will benefit from an investment in our common stock only if it appreciates in value. Investors seeking dividend income should not purchase shares of our common stock.

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

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

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

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

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law as currently in effect may make a change in control of our Company more difficult, even if a change in control may be beneficial to the stockholders.

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

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

**ITEM 1B: UNRESOLVED STAFF COMMENTS**

None.

**ITEM 2: PROPERTIES**

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

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

**ITEM 3: LEGAL PROCEEDINGS**

We know of no material, active or pending legal proceedings against us, or any of our property, and we are not involved as a plaintiff in any material proceedings or pending litigation. There are no proceedings in which any of our directors, officers or affiliates, or any registered or beneficial stockholders are an adverse party or have a material interest adverse to us.

**ITEM 4: MINE SAFETY DISCLOSURES**

Not applicable.

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## PART II

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

## Market Information

In connection with the closing of the 2009 Merger, our common stock commenced trading on the NASDAQ Capital Market on September 30, 2009, under the ticker symbol "RPTPD" with 18,822,162 shares outstanding. Effective October 27, 2009, our ticker symbol changed to "RPTP." Effective February 29, 2012, our common stock commenced trading on the NASDAQ Global Market. As of February 28, 2014, there were 62,479,286 shares of our common stock outstanding. There is no public trading market for our warrants. The closing price for our common stock on February 28, 2014 was \$15.83 per share.

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

	High	Low
Fiscal Year Ended December 31, 2013:		
First Quarter (January 1 – March 31, 2013)	\$6.28	\$4.71
Second Quarter (April 1 – June 30, 2013)	10.47	5.40
Third Quarter (July 1 – September 30, 2013)	15.00	9.26
Fourth Quarter (October 1 – December 31, 2013)	15.29	11.09
Four Months Ended December 31, 2012:		
First Quarter (September 1 – November 30, 2012)	5.74	4.35
December 1, 2012 – December 31, 2012	6.04	5.06
Fiscal Year Ended August 31, 2012:		
First Quarter (September 1 – November 30, 2011)	5.52	3.92
Second Quarter (December 1, 2011 – February 29, 2012)	7.90	5.35
Third Quarter (March 1 – May 31, 2012)	7.31	5.17
Fourth Quarter (June 1 – August 31, 2012)	6.15	4.35
Fiscal Year Ended August 31, 2011:		
First Quarter (September 1 – November 30, 2010)	4.00	2.76
Second Quarter (December 1, 2010 – February 28, 2011)	4.04	3.23
Third Quarter (March 1 – May 31, 2011)	5.75	3.10
Fourth Quarter (June 1 – August 31, 2011)	6.99	3.66

## Holders of Record

As of February 28, 2014, there were approximately 139 holders of record of our common stock. Additionally, on such date, options held by 128 persons to acquire up to, in the aggregate, 9,532,948 shares and warrants held by 7 persons to acquire up to, in the aggregate, 334,764 shares of our common stock, were outstanding.

## Dividends

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

to pay future cash dividends may be restricted by the terms of any future financing.

Recent Sales of Unregistered Securities

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

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## Purchase of Equity Securities and Affiliated Purchasers

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

## Performance Graph

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

The following graph shows the value of an investment of \$100 on September 30, 2009 (date we effected our 2009 Merger) in our common stock, the NASDAQ Composite Index (U.S.), NASDAQ Benchmark TR Index (U.S.) and the NASDAQ Biotechnology Index. All values assume reinvestment of the pretax value of dividends paid by companies included in these indices and are calculated as of August 31, 2010, 2011, 2012, for the 4 months ended December 31, 2012 and as of our year ended December 31, 2013. Our common stock is traded under the ticker symbol RPTP on the NASDAQ Global Market. The comparisons shown in the graph are based upon historical data and we caution that the stock price performance shown in the graph is not indicative of, nor intended to forecast, the potential future performance of our stock.

	September 30, 2009	August 31, 2010	2011	2012	Four months ended December 31, 2012	December 31, 2013
Raptor Pharmaceutical Corp.	\$ 100	\$90.30	\$143.33	\$150.61	\$177.27	\$394.55
NASDAQ U.S. Composite Index	100	100.54	124.79	152.68	151.02	210.35
NASDAQ U.S. Benchmark TR Index	100	101.96	120.68	141.52	145.47	194.18
NASDAQ Biotechnology Index	100	96.73	119.13	168.82	170.41	282.22

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## ITEM 6: SELECTED FINANCIAL DATA

The following table shows selected historical consolidated financial and operating information for, and as of the end of, each of the periods indicated and should be read in conjunction with the information in the sections titled, "Management's Discussion and Analysis of Financial Condition and Results of Operation" and "Business" and our consolidated financial statements and the corresponding notes to those consolidated financial statements included elsewhere in this Annual Report on Form 10-K. The following tables set forth our consolidated balance sheet data as of December 31, 2013 and 2012 and as of August 31, 2012, 2011, 2010 and 2009 and its consolidated statements of operations and comprehensive loss data for the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal years ended August 31, 2012, 2011, 2010 and 2009.

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

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

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

Overview

You should read the following discussion in conjunction with our consolidated financial statements as of December 31, 2013, and the notes to such consolidated financial statements included elsewhere in this Annual Report on Form 10-K. The "Management's Discussion and Analysis of Financial Condition and Results of Operations" section contains forward-looking statements. Please see "Forward-Looking Statements" for a discussion of the uncertainties, risks and assumptions associated with these statements. Our actual results and the timing of certain events could differ materially from those anticipated in these forward-looking statements as a result of certain factors, including those discussed below and elsewhere in this Annual Report on Form 10-K, particularly under the heading "Risk Factors."

Change in Fiscal Year End

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

Plan of Operation and Overview

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

Clinical Development Programs

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

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

Preclinical Product Candidates

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



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### Future Activities

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

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

### Application of Critical Accounting Policies

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

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

### Revenue Recognition and Accounts Receivable

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

PROCYSBI is currently only available for distribution from our U.S. specialty pharmacy partner, the Accredo Health Group, Inc., or Accredo, which is currently our only customer and ships directly to patients. Our distributor in the EU will be the Almac Group, Ltd. for the commercial launch in the EU anticipated to occur in the first half of 2014.

PROCYSBI is not available in retail pharmacies. Prior authorization of coverage level by patients' private insurance plans, our patient assistance program, or PAP, or government payors is a prerequisite to the shipment of PROCYSBI to patients. Revenue is recognized once the product has been shipped by the specialty pharmacy to patients because at this time, we are unable to reasonably estimate rebate percentages based upon our lack of sufficient historical data.

Billings to our distributor in advance of product shipment and delivery by the specialty pharmacy to patients are recorded as deferred revenue by us until such shipments to patients occur.

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

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

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### Inventories and Cost of Sales

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

### Note Payable

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

### Impairment of Goodwill and Intangible Assets

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

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

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

### Common Stock Warrant Liabilities

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

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

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We reported a net loss of \$69.4 million for the year ended December 31, 2013. If our December 31, 2013 closing stock price had been 10% lower, our net loss would have been approximately \$0.9 million lower. If our December 31, 2013 closing stock price had been 10% higher, our net loss would have been approximately \$0.9 million higher. If our December 31, 2013 volatility assumption had been 10% lower, our net loss would have been approximately \$0.1 million lower. If our December 31, 2012 volatility assumption had been 10% higher, our net loss would have been approximately \$0.1 million higher.

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

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

### Income Taxes

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

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

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

### Stock-Based Compensation

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

We based our Black-Scholes inputs on the following factors: the risk-free interest rate was based upon our review of current constant maturity treasury bill rates for five years; the expected life of five years was based upon our assessment of the ten-year term of the stock options issued along with the fact that we are a development-stage company and our anticipation that option holders will exercise stock options when we are at a more mature stage of development; the volatility was based on a combination of the actual annualized volatility of our common stock price as quoted on NASDAQ since the closing of our 2009 Merger on September 30, 2009 and of annualized volatility of peer companies; the turnover rate was based on our assessment of our historical employee turnover; and the dividend rate was based on our current decision to not pay dividends on our stock at our current corporate stage of development. If actual results differ significantly from these estimates, stock-based compensation expense and results of operations could be materially impacted. See Note 8 of our consolidated financial statements for a further discussion of our accounting for stock-based compensation.

Table of ContentsResults of Operations  
For the year ended December 31, 2013

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

## Revenue

We recognized \$10.2 million in PROCYSBI net product sales for the fourth quarter of 2013. Net product sales for the year 2013 totaled \$16.9 million. There were no product sales for the comparable prior periods as PROCYSBI became commercially available in the U.S. in June 2013.

## Cost of Sales

Prior to the approval of PROCYSBI by the FDA on April 30, 2013 and in Europe, prior to EMA approval on September 6, 2013, we recorded manufacturing costs relating to PROCYSBI as research and development expense. Subsequent to approval, we began capitalizing these costs as commercial inventory. PROCYSBI became commercially available in mid-June 2013, and we began recognizing manufacturing costs as capitalized inventory. Costs capitalized as inventory are expensed as cost of sales as product is sold. During the year ended December 31, 2013, we recorded a \$0.4 million reserve as cost of sales representing commercial inventory that was capitalized subsequent to FDA approval but written off due to an unanticipated minor change in the finished product presentation. Cost of sales includes the cost of inventory sold, inventory and reserve costs, manufacturing and supply chain costs, product shipping and handling costs, amortization of licensing approval milestone payments and licensing royalties payable to UCSD.

## Research and Development

For the year ended December 31, 2013, our research and development expenses consisted primarily of costs associated with the manufacturing and testing of clinical and commercial materials in anticipation for our potential launch of RP103 for cystinosis, clinical trial research expenses and employee compensation. The increase in research and development expenses relates primarily to increased clinical product manufacture of RP103 for the potential treatment of cystinosis (prior to the capitalization of such amounts upon FDA and EMA approval); HD, NASH, cystinosis extension, and other supporting study expenses and related employee compensation, offset by a reduction in Phase 3 cystinosis clinical trial expenses.

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Major Program expenses recorded as research and development:

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

#### Selling, General and Administrative Expenses

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

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

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

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

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## Current Status of Major Programs

Please refer to the Item 1 of this Annual Report on Form 10-K for a detailed discussion of each of our major programs. We currently have product candidates in clinical development as potential treatments for HD, NAFLD, Leigh syndrome and other mitochondrial disorders and ALDH2. Our preclinical programs are based upon bioengineered novel drug candidates that are designed to target cancer and other diseases. We continue efforts to out-license Convivia and our preclinical programs.

Any of our major programs could be partnered for further development and/or could be accelerated, slowed or ceased due to scientific results or challenges in obtaining funding. We anticipate that we will need additional funding in order to pursue our plans beyond the next 18 months. In addition, the timing and costs of development of our programs beyond the next 18 months is highly uncertain and difficult to estimate. See Part I, Item 1A of this Annual Report on Form 10-K titled "Risk Factors" for further discussion about the risks and uncertainties pertaining to drug development.

## Interest Expense

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

## Adjustment to the Fair Value of Common Stock Warrants

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

For the four months ended December 31, 2012 and 2011

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

## Research and Development

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

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

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Major Program expenses recorded as research and development:

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

#### General and Administrative Expenses

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

Major Program expenses recorded as general and administrative expenses:

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

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

Additional major program expenses include patent fees and patent expenses which were recorded as general and administrative expenses as these fees are to support patent applications (not issued patents) and expenses related to the pre-commercial launch of RP103 for the potential treatment of cystinosis.

Adjustment to the Fair Value of Common Stock Warrants

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

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Years ended August 31, 2012 and 2011

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

## Research and Development

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

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

Research and development expenses include the following:

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

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

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## General and Administrative Expenses

General and administrative expenses include finance, executive and sales and marketing compensation and benefits, pre-commercial expenses, such as reimbursement and marketing studies, corporate costs, such as legal and auditing fees, business development expenses, travel, board of director fees and expenses, investor relations expenses, intellectual property costs associated with filed (but not issued) patents, administrative consulting and allocated human resources and facilities costs. General and administrative expenses for the year ended August 31, 2012 increased by approximately \$8.6 million compared to the prior fiscal year. The increase was primarily due to:

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

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

## Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants was a loss of approximately \$3.2 million for the year ended August 31, 2012 compared to a loss of approximately \$16.3 million for the year ended August 31, 2011, a decrease in loss of approximately \$13.1 million which resulted primarily from the lower number of remaining warrants outstanding due to warrant exercises.

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Liquidity and Capital Resources

Capital Resources

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

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

On April 30, 2012, we entered into a Sales Agreement with Cowen and Company, or Cowen, to sell shares of our common stock, with aggregate gross sales proceeds of up to \$40 million, from time to time through an "at the market" equity offering program under which Cowen acts as sales agent. We pay a 3% commission to Cowen on any sales pursuant to this Sales Agreement.

On July 3, 2013, we amended and restated the Sales Agreement to increase the aggregate gross sales proceeds that may be raised to \$100 million. Cumulatively through December 31, 2013, we sold 7,599,474 shares under the ATM offerings at a weighted-average selling price of \$7.08 per share for net proceeds of approximately \$52.1 million. As of February 28, 2014, 334,764 shares (including the placement agent warrant described below) of our common stock warrants were outstanding, all of which warrants were issued pursuant to private placement purchase agreements, dated as of August 9, 2010.

Future Funding Requirements

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

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

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

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

### Research and Development Activities

We plan to conduct further research and development, to support several clinical trials for RP103, improve upon our internal discovery molecules and in-licensed technology and continue business development efforts for our preclinical and other clinical-stage product candidates in the next 12 months. We plan to conduct research and development activities by our own laboratory staff and also by engaging contract research organizations, clinical research organizations and contract manufacturing organizations. We also plan to incur costs for the production of our clinical study drug candidate RP103 for the potential treatment of HD and NASH; for production of RP103 for additional clinical trials in cystinosis; clinical and medical advisors; and consulting and collaboration fees. We anticipate that our research and development costs will increase during the next 12 months primarily due to the addition of new studies in support of cystinosis, HD, NAFLD, Leigh syndrome and other indications.

### Selling, General and Administrative Activities

Selling, general and administrative costs in the next 12 months will consist primarily of sales activities surrounding the sale of PROCYSBI in the U.S. and the commercial launch of PROCYSBI in the EU, of legal, tax and accounting fees, patent legal fees, investor relations expenses, board fees and expenses, insurance, rent and facility support expenses. We anticipate that selling, general and administrative expenses will continue to increase in support of PROCYSBI sales growth, as well as an increase in facilities and administrative expenses to support our rapid growth.

### Capital Expenditures

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

### Contractual Obligations

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

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

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## Other Contractual Obligations

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

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

We maintain several contracts with contract manufacturers, clinical organizations and clinical sites, drug labelers and distributors and research organizations, primarily to assist with clinical research and clinical manufacturing for our cystinosis and HD programs and our NAFLD clinical collaboration. The future commitments pursuant to these agreements, some of which include estimates of amounts or timing of payments, are included in the table above as research and development and purchase commitments.

We are also subject to contingent payments related to various development activities totaling approximately \$14.9 million, which are primarily due upon the achievement of certain development and commercial milestones if such milestones occur before certain dates in the future. These contingent payments are not included in the table above as we cannot reliably predict their timing or occurrence.

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

## Off-Balance Sheet Arrangements

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

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ITEM 7A: QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Market risk represents the risk of changes in the value of market risk sensitive instruments caused by fluctuations in interest rates, foreign exchange rates and commodity prices. We are exposed to various market risks that may arise from adverse changes in market rates and prices, such as foreign exchange rate and interest rate fluctuations. We do not enter into derivatives or other financial instruments for trading or speculative purposes.

Foreign Exchange Risk

A majority of our assets and liabilities are maintained in the U.S. in U.S. dollars and a majority of our expenditures are transacted in U.S. dollars. We are subject to foreign exchange risk for the operations of BV, SAS, GmbH and CV, which use the Euro as their functional currency. We do not believe that a hypothetical one percentage point fluctuation in the U.S. dollar exchange rate would materially affect our consolidated financial position, results from operations or cash flows as of December 31, 2013. We do not currently hedge our foreign currency transactions.

Interest Rate Risk

As of December 31, 2013, we had approximately \$70.6 million in cash equivalent money market accounts, yielding approximately 0.05% per year. A hypothetical one percentage point decline in interest rates would not have materially affected our consolidated financial position, results of operations or cash flows as of December 31, 2013.

ITEM 8: FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

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

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

Financial Statements

	Page
<u>Reports of Independent Registered Public Accounting Firm</u>	91
<u>Report of Former Independent Registered Public Accounting Firm</u>	93
<u>Consolidated Balance Sheets as of December 31, 2013 and December 31, 2012</u>	94
<u>Consolidated Statements of Operations and Comprehensive Loss for the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal years ended August 31, 2012 and 2011</u>	95
<u>Consolidated Statements of Stockholders' Equity (Deficit) for the fiscal years ended August 31, 2011 and 2012, the four months ended December 31, 2012 and the year ended December 31, 2013</u>	96
<u>Consolidated Statements of Cash Flows for the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal years ended August 31, 2012 and 2011</u>	98
<u>Notes to Consolidated Financial Statements</u>	100

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PART II – FINANCIAL INFORMATION

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

None.

ITEM 9A: CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

As of December 31, 2013, we performed an evaluation of the effectiveness of our disclosure controls and procedures that are designed to ensure that the information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act, is accumulated and communicated to our management, including our principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Our disclosure controls and procedures were designed to provide reasonable assurance of achieving our control objectives. Based on our evaluation, our management, including our Chief Executive Officer and Chief Financial Officer, has concluded that our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Exchange Act) were effective at a reasonable assurance level as of December 31, 2013.

Management's Report on Internal Control Over Financial Reporting

Our management, under the supervision of our Chief Executive Officer and Chief Financial Officer, is responsible for establishing and maintaining adequate internal control over our financial reporting, as defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act. Our internal control over financial reporting is defined as a process designed by, or under the supervision of, our Chief Executive Officer and Chief Financial Officer, and effected by our board of directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP. Internal control over financial reporting includes policies and procedures that: (i) pertain to the maintenance of records that in reasonable detail accurately and fairly reflect our transactions and asset dispositions; (ii) provide reasonable assurance that transactions are recorded as necessary to permit the preparation of our financial statements in accordance with GAAP, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and (iii) provide reasonable assurance regarding the prevention or timely detection of unauthorized acquisition, use or disposition of assets that could have a material effect on our financial statements. Under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework in the 1992 Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on our evaluation under the framework in Internal Control-Integrated Framework, our management concluded that our internal control over financial reporting was effective as of December 31, 2013.

Limitations on the Effectiveness of Controls

Due to its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Attestation Report of Grant Thornton LLP

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

Changes in Internal Control over Financial Reporting

During the quarter ended December 31, 2013, there have not been any material changes in our internal control over financial reporting or in other factors that have materially affected, or are reasonably likely to materially affect, our

internal control over financial reporting.

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ITEM 9B: OTHER INFORMATION

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

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## PART III

## ITEM 10: DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

## Directors

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

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

(1) Member of the Corporate Governance and Nominating Committee.

(2) Member of the Audit Committee.

(3) Member of the Compensation Committee.

## Business Experience and Directorships

The following describes the background of our directors.

Raymond W. (Bill) Anderson. Mr. Anderson has served as a director of the Company since September 2009 and has more than 30 years of biopharmaceutical/medical technology sector experience, primarily focused in financial management. Mr. Anderson worked at Dow Pharmaceutical Sciences, Inc. (now a wholly owned subsidiary of Valeant Pharmaceuticals International) from July 2003 until he retired in June 2010. He most recently served as Dow's Managing Director since January 2009 and previously served as Chief Financial Officer and Vice President, Finance and Administration. Prior to joining Dow in 2003, Mr. Anderson was Chief Financial Officer for Transurgical, Inc., a private medical technology company. Prior to that, Mr. Anderson served as Chief Operating Officer and Chief Financial Officer at BioMarin Pharmaceutical Inc. from June 1998 to January 2002. Prior to June 1998, Mr. Anderson held similar executive-level positions with other biopharmaceutical companies, including Syntex Laboratories, Chiron Corporation, Glycomed Incorporated and Fusion Medical Technologies. Mr. Anderson also served as an officer in the United States Army Corps of Engineers, as a strategic planner and operational profit and loss manager at General Electric and as a finance manager at Memorex. Mr. Anderson holds an M.B.A. from Harvard University, an M.S. in Administration from George Washington University and a B.S. in Engineering from the United States Military Academy. We nominated Mr. Anderson to the Board of Directors primarily due to his 30 years of healthcare experience in the areas of operations and finance.

Suzanne L. Bruhn, Ph.D. Dr. Bruhn has served as a director of the Company since April 2011. She is currently President and Chief Executive Officer of Promedior, Inc., a privately held, clinical-stage biotechnology company focused on the development of targeted therapeutics to treat diseases involving fibrosis. Immediately prior to her appointment as Promedior's Chief Executive Officer, Dr. Bruhn spent 13 years at Shire Human Genetic Therapies (HGT), a division of Shire plc, specializing in the development and commercialization of treatments for orphan diseases. Dr. Bruhn's most recent position at Shire HGT was Senior Vice President, Strategic Planning and Program Management. At Shire HGT, Dr. Bruhn was responsible for establishing the program management function, driving strategic planning and portfolio management, and for global regulatory affairs. Dr. Bruhn played a key role in the development, registration and global expansion of Shire HGT's products REPLAGAL®, ELAPRASE® and VPRIV®. She also played a key role in Shire HGT's portfolio expansion through acquisitions, including the acquisition of FIRAZYR®. Prior to her time at HGT, Dr. Bruhn held various positions at Cytotherapeutics, Inc., a biotechnology company. Dr. Bruhn holds a Ph.D. in Chemistry from Massachusetts Institute of Technology and was a

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

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Richard L. Franklin, M.D., Ph.D. Dr. Franklin has served as a director of the Company since September 2009. Dr. Franklin has served as the Chief Executive Officer and a director of Tarix Pharmaceuticals, a drug development company, since 2004. He has also served as the Chairman of Pathfinder, LLC, a regenerative medicine company, since 2009. Dr. Franklin served as Chairman of the board of directors of SyntheMed, Inc., a biomaterials company engaged in the development and commercialization of medical devices, from June 2003 to September 2011, and as a director of that company from December 2000 to September 2011. Pathfinder, LLC and SyntheMed, Inc. merged in September 2011, at which point the combined companies were renamed Pathfinder Cell Therapy, Inc., and Dr. Franklin became the Chief Executive Officer and a director of the surviving entity. Dr. Franklin received an M.A. in Mathematics from University of Wisconsin, a Ph.D. in Mathematics from Brandeis University and an M.D. from Boston University School of Medicine. We nominated Dr. Franklin to the Board of Directors due to his experience as a CEO and chairman of various healthcare companies.

Llew Keltner, M.D., Ph.D. Dr. Keltner has served as Chairman of the Board of the Company since July 2013 and as a director since September 2009. Dr. Keltner is Chief Executive Officer of EPISTAT, an international healthcare technology transfer, corporate risk management and healthcare strategy company that he founded in 1972. Dr. Keltner also served as the Chief Executive Officer of AgonOx, a biotech company developing OX40 agonists for use in cancer therapy, from 2011 to 2013. From 2010 until 2011, Dr. Keltner was the President of Novici Biotech, a privately held gene and protein optimization firm, and from 2001 to 2010, he was Chief Executive Officer and President of Light Sciences Oncology, a privately held biotechnology company developing a late-stage, light-activated therapy for hepatocellular cancer and other solid tumors. From 1997 to 2004, Dr. Keltner was Chief Executive Officer of Metastat, Inc., a development-stage biotech company focused on cancer metastasis. Dr. Keltner holds positions on the boards of Infostat, BioQuiddity, Oregon Life Sciences and Goodwell Technologies. He previously served as a director of Light Sciences Corporation, Vital Choice, Thesis Technologies, Oread Companies and MannKind Corporation. Dr. Keltner has also been a scientific advisory board member at Lifetime Corporation, ASB Meditest, Oread Laboratories, Hall-Kimbrell and AAIPharma. Dr. Keltner is an Associate Professor at Case Western Reserve School of Medicine and a Guest Lecturer and Director in the Bioethics Program at Columbia University School of Medicine. He is currently a member of the American Society of Clinical Oncology, the American Medical Association, the International Association of Tumor Marker Oncology, the American Association of Clinical Chemistry and the Drug Information Association. Dr. Keltner received an M.S. in Epidemiology and Biostatistics, a Ph.D. in Biomedical Informatics and an M.D. from Case Western Reserve University in Cleveland, Ohio. Dr. Keltner has also authored many research publications. We nominated Dr. Keltner to the Board of Directors due to his practical experience as a chief executive officer of a life sciences company and due to his medical knowledge and network within the biotechnology industry.

Erich Sager. Mr. Sager has served as a director of the Company since September 2009 and served as the Chairman of our Board of Directors from September 2009 through July 2013. Mr. Sager was a founding partner of Limetree Capital SA, a Swiss-based investment banking boutique, where he served as Chairman from 2006 to 2011. Mr. Sager currently serves as Chairman and a member of the board of directors at Calltrade Carrier Services AG, a European wholesale phone operator, and has held such position since 2004. He is also a current board member of Zecotek Photonics Inc. and Pulse Capital Corp. Mr. Sager served on the board of directors of BioMarin from November 1997 to March 2006 and as chairman of LaMont Asset Management SA, a private investment management firm, from September 1996 until August 2004. Mr. Sager has also held various positions at banks in Switzerland, including Senior Vice President, Head of the Private Banking for Dresdner Bank (Switzerland) Ltd. and Vice President, Private Banking, Head of the German Desk for Deutsche Bank (Switzerland) Ltd. Mr. Sager received a business degree from the School of Economics and Business Administration, Zurich, Switzerland. We nominated Mr. Sager to the Board of Directors due to his knowledge of healthcare fundraising in Europe, as well as his experience while at BioMarin.

Vijay B. Samant. Mr. Samant has served as a director of the Company since April 2011. He currently serves as President and Chief Executive Officer of Vical Inc., a publicly traded company focused on the development of DNA vaccines for infectious diseases and cancer therapeutics. Prior to his time at Vical, Mr. Samant spent more than 20 years in diverse U.S. and international sales, marketing, operations and business development positions with Merck &

Company, Inc., including Vice President of Vaccine Operations, Vice President of Business Affairs and Executive Director of Materials Management, all in the Merck Manufacturing Division, and Chief Operating Officer of the Merck Vaccine Division. Mr. Samant served as a member of the Board of Trustees for the International Vaccine Institute (IVI, Seoul, Korea) from 2008 to 2012, a member of the Board of Trustees for the National Foundation for Infectious Diseases (NFID, Bethesda, MD) from 2003 to 2012 and a Director of the Aeras Global TB Vaccine Foundation from 2001 to 2010. Mr. Samant holds an S.M. from the Sloan School of Management at the Massachusetts Institute of Technology, as well as an M.S. in Chemical Engineering from Columbia University and a B.S. in Chemical Engineering from the University of Bombay, University Department of Chemical Technology. We nominated Mr. Samant to the Board of Directors due to his experience in running a public healthcare company and due to his background in sales and marketing and business development.

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Christopher M. Starr, Ph.D., Chief Executive Officer. Dr. Starr has served as the Chief Executive Officer and a director of Raptor Pharmaceutical Corp. since September 2009. Dr. Starr was a co-founder of RPC and has served as the Chief Executive Officer, President and director thereof since its inception in 2006. Dr. Starr has served as Chief Executive Officer of our wholly owned subsidiary, Raptor Pharmaceutical Inc., since its inception in September 2005. Dr. Starr co-founded BioMarin Pharmaceutical Inc. in 1997 where he last served as Senior Vice President and Chief Scientific Officer prior to joining us in 2006. As Senior Vice President at BioMarin, Dr. Starr was responsible for managing a Scientific Operations team of 181 research, process development, manufacturing and quality personnel through the successful development of commercial manufacturing processes for its enzyme replacement products, and supervised the cGMP design, construction and licensing of BioMarin's proprietary biological manufacturing facility. From 1991 to 1998, Dr. Starr supervised research and commercial programs at BioMarin's predecessor company, Glyko, Inc., where he served as Vice President of Research and Development. Prior to his tenure at Glyko, Inc., Dr. Starr was a National Research Council Associate at the National Institutes of Health. Dr. Starr earned a B.S. from Syracuse University and a Ph.D. in Biochemistry and Molecular Biology from the State University of New York Health Science Center, in Syracuse, New York. We nominated Dr. Starr to the Board of Directors due to his extensive experience at BioMarin Pharmaceutical where he was directly involved in the successful approval of two drugs for orphan indications.

Timothy P. Walbert. Mr. Walbert has served as a director of the Company since April 2011. He is currently the Chairman, President and Chief Executive Officer of Horizon Pharma, Inc., a publicly traded biopharmaceutical company focused on developing and commercializing innovative medicines in arthritis, pain and inflammatory diseases. Prior to his time at Horizon Pharma, Mr. Walbert served as President, Chief Executive Officer and a director of IDM Pharma, Inc., a publicly traded oncology-focused biotechnology company, which was acquired by Takeda Pharma Holdings in June 2009. For more than 20 years, Mr. Walbert held executive positions in general management, corporate strategy, sales, U.S. and international marketing and commercial operations at biopharmaceutical companies such as Abbott Laboratories, G.D. Searle/Pharmacia, Neopharm, Merck & Company and Wyeth. At Abbott, Mr. Walbert served as Divisional Vice President and General Manager, Immunology, leading the global development and launch of HUMIRA, which attained over \$8.0 billion in sales in 2011. Mr. Walbert serves on the board of directors of XOMA Ltd., the Biotechnology Industry Organization (BIO), the Illinois Biotechnology Industry Organization (iBIO) and the Greater Chicago Arthritis Foundation. Mr. Walbert holds a B.A. in Business and Marketing from Muhlenberg College. We nominated Mr. Walbert to the Board of Directors due to his experience in commercial operations and business strategy and his experience leading a publicly traded biopharmaceutical company.

**Audit Committee**

The audit committee of our board of directors, herein referred to as the Audit Committee, has been established in accordance with Section 3(a)(58)(A) of the Exchange Act. The Audit Committee is responsible for overseeing our accounting and financial reporting processes. In such capacity, our Audit Committee (a) has sole authority to appoint, replace and compensate our independent registered public accounting firm and is directly responsible for oversight of its work; (b) approves all audit fees and terms, as well as any permitted non-audit services performed by our independent registered public accounting firm; (c) meets and discusses directly with our independent registered public accounting firm its audit work and related matters; (d) oversees and performs investigations with respect to our internal and external auditing procedures, including the receipt, retention and treatment of complaints received by us regarding accounting, internal accounting controls or auditing matters and the confidential and anonymous submission by employees of concerns regarding questionable accounting or auditing matters and (e) undertakes such other activities as the Audit Committee deems necessary or advisable and as may be required by applicable law.

Our Audit Committee currently consists of Mr. Anderson (Chair), Dr. Franklin, Mr. Sager and Mr. Walbert. Mr. Anderson has been designated as the "audit committee financial expert" as defined by the regulations promulgated by the SEC. Our Board of Directors has determined that each member of the Audit Committee is independent as defined by NASDAQ and SEC rules applicable to audit committee members.

Section 16(a) Beneficial Ownership Reporting Compliance

Section 16(a) of the Exchange Act requires our directors, executive officers and holders of more than ten percent of a registered class of our equity securities, or 10% stockholders, to file reports of ownership and reports of changes in ownership of our common stock and other equity securities with the SEC. Directors, executive officers and 10% stockholders are required to furnish us with copies of all Section 16(a) forms they file. Based solely on a review of the copies of such reports furnished to us, we believe that during the year ended December 31, 2013, our directors, executive officers and 10% stockholders timely filed all Section 16(a) reports applicable to them.

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## Code of Ethics

We have adopted a Code of Business Conduct and Ethics, which is applicable to our directors and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller or persons performing similar functions. Our Code of Business Conduct and Ethics is posted in the Corporate Governance section of our website at [www.raptorpharma.com](http://www.raptorpharma.com) and is acknowledged by our executive officers and directors on an annual basis. We intend to satisfy the disclosure requirements under Item 5.05 of the SEC Form 8-K regarding an amendment to, or waiver from, a provision of our Code of Business Conduct and Ethics by posting such information on our website at the website address and location specified above.

## Executive Officers

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

Our executive officers are elected by our Board of Directors on an annual basis and serve at the discretion of our Board of Directors or until their successors have been duly elected and qualified. Below is a current list of the executive officers of our Company.

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

Julie Anne Smith. Ms. Smith has served as our Executive Vice President, Strategy and Chief Operations Officer since September 2012. Ms. Smith is responsible for directing our commercial, manufacturing and program management organizations and providing leadership in corporate and strategic development initiatives. In her nearly 20 years in biotechnology, Ms. Smith has served in executive management of both privately held and publicly held biotechnology firms, mostly in orphan drug development and commercial product opportunities. Prior to joining us, from July 2008 to May 2012, Ms. Smith was Chief Commercial Officer of Enobia Pharma, Inc., a privately held, clinical-stage orphan drug company later acquired by Alexion. From August 2006 to July 2008, she led commercial functions as Vice President, Commercial at Jazz Pharmaceuticals. From December 2001 to August 2006, as Vice President, Global Marketing at Genzyme General in Cambridge MA, she led the worldwide commercialization and planning for Myozyme, an infused enzyme replacement therapy for an ultra-orphan genetic disease. Ms. Smith holds a B.S. in Biological and Nutritional Science from Cornell University, Ithaca, New York.

Georgia Erbez. Ms. Erbez has served as our Chief Financial Officer, Treasurer and Secretary since September 2012. Ms. Erbez is responsible for directing our global financial strategy and organization and provides leadership in defining, communicating and executing corporate and financial strategic initiatives. Prior to joining us, from March 2008 to September 2012, Ms. Erbez was a founder and Managing Director of Beal Advisors, a boutique investment bank providing advisory and capital acquisition services to emerging growth companies. Ms. Erbez also served as Managing Director and Consultant at Collins Stewart LLC from April 2011 to January 2012. From 2005 to 2008, Ms. Erbez was a Senior Vice President in the life sciences investment banking group at Jefferies & Co. From 1998 to 2002, she was with the healthcare investment banking group at Cowen and Co., most recently as Director. From 1997 to 1998, Ms. Erbez was an associate at Hambrecht & Quist, where she provided investment banking services to life sciences companies and healthcare services. From July 1989 to January 1997, Ms. Erbez was with Alex Brown & Sons in the healthcare investment banking group, where she focused on life sciences, medical technology and healthcare services companies. Ms. Erbez holds a B.A. in International Relations with an emphasis in Economics from the University of California at Davis.

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

raise over \$50.0 million in venture capital and grew Instill to an operation of more than 150 people with a nationwide customer base. After leaving Instill, from 2001 and 2007, Mr. Daley served in executive and consulting roles for a number of technology startup companies, including MetricStream, Inc., PartsRiver and Certicom Security. Prior to that time, Mr. Daley worked in operations management for Anheuser-Busch, Inc. and consulted to Gordon Biersch Brewing Company and Lion Breweries in New Zealand. Mr. Daley received a B.S. in Fermentation Science from University of California at Davis and an M.B.A. from Stanford University.

Relationships Among Executive Officers and Directors

There are no family relationships among any of our directors or executive officers.

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ITEM 11: EXECUTIVE COMPENSATION

Named Executive Officer Compensation  
Compensation Discussion and Analysis

Overview

The Compensation Committee has overall responsibility for the compensation program for our executive officers. The Compensation Committee reviews, adopts and oversees our compensation strategy, policies, plans and programs, including:

- (i) the establishment of corporate and individual performance goals and evaluation of performance relevant to the compensation of our executive officers and other senior management;
- (ii) the review and approval of the terms of employment or service, including severance and change in control arrangements, of our Chief Executive Officer and the other executive officers;
  - the review and recommendation to the Board of Directors of the compensation plans and programs advisable for
  - (iii) the Company, including the type and amount of compensation to be paid or awarded to non-employee directors; and
- (iv) the administration of our equity compensation plans, pension and profit-sharing plans, deferred compensation plans and other similar plans and programs.

In evaluating executive officer pay, the Compensation Committee may retain the services of an independent compensation consultant or research firm and consider recommendations from our Chief Executive Officer and persons serving in managerial positions over a particular executive officer with respect to goals and compensation of the executive officer. The executive officers are not present or involved in deliberations concerning their compensation. Our Compensation Committee assesses the information it receives in accordance with its business judgment. All decisions with respect to executive compensation are first approved by our Compensation Committee and then submitted, together with the Compensation Committee's recommendations, to our Board of Directors for final approval. Our Chief Executive Officer is not present for the discussion of and approval of his compensation. We choose to pay the various elements of compensation discussed in order to attract, retain and motivate our high quality executive talent, reward annual performance and provide incentive for the achievement of intermediate and long-term strategic goals.

The Compensation Committee's philosophy is to:

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

Our allocation between currently paid cash compensation and longer term equity compensation is intended to balance the requirement for adequate base compensation to attract, retain and motivate highly skilled personnel, while providing equity incentives to maximize long-term value for our stockholders and thus for our employees. We provide

cash compensation in the form of base salary and annual, discretionary incentive cash bonuses to reward performance against preset written goals and objectives. We provide non-cash equity compensation to reward performance against current, intermediate and long-term strategic goals and provide a basis for improved financial security for the employee if our stockholders and we have financial success.

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The Role of Stockholder Say-on-Pay Votes

We provide our stockholders with the opportunity to cast a non-binding advisory vote on the compensation of our named executive officers' and on the frequency with which this vote should be conducted in future years. During the year ended December 31, 2013, our named executive officers included our principal executive officer (Chief Executive Officer), our principal financial officer (Chief Financial Officer), our Chief Operations Officer and our Chief Business Officer. In July 2013 at our Annual Meeting of Stockholders, based upon total shares voted, our stockholders approved our named executive officers' compensation with a 98.9% affirmative vote. Although the stockholder vote is non-binding, the Compensation Committee will consider the outcome of the vote when making future compensation decisions for named executive officers. In addition, we will conduct future stockholder advisory votes on the compensation of our named executive officers once every year, until the next required stockholder advisory vote on the frequency of future stockholder advisory votes on the compensation of our named executive officers, which we will conduct at our 2015 Annual Meeting of Stockholders.

Approach for Determining Form and Amount of Compensation

Use of External Compensation Consultant

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

- market survey data; and
- a comprehensive review of our peer group companies.

Comparison to Market Practices

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

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

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

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

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

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

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

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

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

The Role of Our Chief Executive Officer

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

Elements of Compensation

Elements of compensation for our executives generally include:

- base salary (typically subject to review and potential adjustment annually based on inflation factors, industry competitive salary levels, our ability to pay, and performance on corporate and individual goals);
- annual performance bonuses which are paid in cash and are based primarily on performance against preset written goals;
- equity compensation (which to date has been implemented using stock option awards with multiple year vesting terms and up to ten year expiration periods);
- 401(k) plan Company matching contributions;
- health, disability and life insurance; and

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

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## Base Salary

At hire, base salaries are set for our executives based on the scope of each executive's responsibilities, as well as their qualifications, breadth of experience, performance record in similar situations, depth and breadth of appropriate functional expertise and close match with position requirements. Competitive market compensation paid by similar companies in our industry for individuals with similar responsibilities is a fundamental consideration.

Shortly after the end of each fiscal year, the Compensation Committee conducts an annual review of base salaries and the overall compensation package as a basis for any adjustments. Annual adjustments, if any, are typically made effective retroactive to the first day of the new fiscal year. The basis for salary adjustments may include merit increases in the competitive marketplace, adjustments to move individuals toward our target penetration in the competitive salary range for similar positions, increased duties and responsibilities, and sustained superior performance against goals and in special assignments. Adjustments may be made during the fiscal year for promotions, for highly urgent competitive reasons, for sustained superior performance in new or special challenges or circumstances, and similar reasons (mid-year adjustments generally require unusual or special circumstances). There were no changes made to the base salary compensation of our named executive officers during the 16-month period ended December 31, 2013 as the Compensation Committee had recommended and the Board of Directors had approved the following base salaries effective September 2012:

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

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

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

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

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

All of our executive officers are eligible for annual cash bonuses pursuant to their employment agreements.

Our Compensation Committee has implemented an annual performance program. Annual performance goals are determined and documented in writing at the beginning of each fiscal year for the Company as a whole (corporate goals) and for each executive (individual goals). Should there be a meaningful change in our situation, environment, or operating strategy, goals may be modified or new, more appropriate goals may be instituted upon the recommendation of our Compensation Committee and approval by our Board of Directors. Given that we changed our fiscal year in 2012 from August 31 to December 31, the cash incentive program covered the 16-month period from September 1, 2012 through December 31, 2013.

Performance against our corporate goals and the executive's individual goals is considered by our Compensation Committee in evaluating performance and as a significant contributing factor in determining all aspects of the compensation of our executives. Performance against goals is the primary factor in determining annual cash incentive bonuses.

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Goals are weighted in importance and are time-bound. When taken as a whole, goals are intended to be challenging goals which will have a meaningful impact on stockholder value, either immediately or as preparatory steps required for future achievements.

The achievement scores are desired to be measurable and quantifiable. After evaluation of performance, achievement scores may be awarded which recognize partial performance of a goal or award additional score points for exceptional performance due to unanticipated challenges or superior performance.

Each organizational level in the Company has a target percentage of the annual base salary for annual incentive bonus awards. Such awards are granted at the sole discretion of our Board of Directors, include subjective aspects of performance, and can be modified based on multiple factors including our available financial resources, our overall performance and others. Bonuses are pro-rated for the time of service within the year. An employee must still be in active service at the time our Board of Directors determines to be eligible to be paid an annual incentive bonus. Awards can vary up to 125% of the target percentage based on assessment of the achievement of meaningful additional goals or sustained superior performance in the conduct of duties and responsibilities in the employee's position.

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

Corporate Goals

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

Development of RP103 for Cystinosis (weighted 70%; achieved 60%)

- NDA approval on PDUFA date;
- MAA approval Q3 calendar 2013;
- Meet patient enrollment goals for RaptorCares™ in U.S. and EU;
- Launch in the U.S. as per schedule and achieve meaningful net revenue goals; and
- Obtain orphan exclusivity from FDA/EMA.

Finance (weighted 25%; achieved 25%)

- End 2013 with cash balance of \$84 million; and
- Meet or exceed EBITDA goal of -\$54 million for calendar 2013.

RP103 – Other (weighted 5%; achieved 5%)

- Contract with second source supplier.

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

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### Individual Goals

Our Chief Executive Officer's individual goals are identical to the corporate goals. Individual goals are proposed by each executive and reviewed by our Chief Executive Officer. After review and modification, if necessary, by our Compensation Committee, the goals are approved by our Board of Directors.

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

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

### Fiscal Year 2013 Goal Achievements for our Executive Officers

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

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

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

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

### Equity Incentive Programs (Currently Based on Stock Options)

We believe that equity grants provided to our executive officers (and all members of our team) create a strong link to our long-term financial and equity market performance, create an ownership culture and closely align the interests of our executive officers with the interests of our stockholders. Because of the direct relationship between the value of an equity award and the future market price of our common stock, we believe that granting equity awards is the best method of motivating executive officers to manage in a manner that is consistent with our stockholders' and our Company's interests. In addition, we believe that the four-year vesting feature of our equity grants promotes executive officer (and staff) retention because this feature provides an incentive of potentially increasing value to our executive officers during the vesting period.

In determining the size of equity grants to our executive officers, our Compensation Committee considers: our performance; the applicable executive officer's performance; comparative competitive levels of equity compensation for similar peer companies; the vesting of such awards; the number of shares available under our 2010 Equity Incentive Plan, or the 2010 Plan, and projected future needs to support future staff growth; the recommendations of management and consultants; and external data sources which support a comparative competitive analyses.

With respect to newly hired executives, our practice is to include equity compensation (currently based on stock option grants) as an integral part of the compensation package for inclusion in the executive's employment agreement. The compensation package, including the stock option grant, is approved by a unanimous written consent executed by our Board of Directors. The executive's stock option exercise price is based upon the closing price the day preceding the later of approval by the Board of Directors or the executive's first day of employment.

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Under the 2010 Plan, we were initially authorized to grant up to an aggregate of 3,000,000 shares over the ten year life of the 2010 Plan. On April 7, 2011, our stockholders passed amendments to the 2010 Plan which allow for an increase of the grant pool based upon 5% of our common stock outstanding as of April 7, 2011, August 31, 2011 and August 31, 2012 up to an aggregate maximum increase of 6,000,000 shares. The April 7, 2011, August 31, 2011 and August 31, 2012 increases added 1,629,516, 1,778,459 and 2,528,407 shares, respectively, available for grant under the 2010 Plan. On July 23, 2013, our stockholders approved an amendment to the 2010 Plan to increase the share reserve by an additional 3,000,000 shares. As of December 31, 2013, options to purchase 8,217,674 shares of our common stock were outstanding and 3,861,729 shares of our common stock remained available for future issuance under the 2010 Plan.

**Stock Options.** Stock options granted by us have an exercise price equal to the fair market value of our common stock on the day prior to grant, typically vest over a four-year period with 6/48ths vesting six months after the vesting commencement date and the remainder vesting ratably each month thereafter based upon continued employment or service, and generally expire ten years after the date of grant. Incentive stock options also include certain other terms necessary to assure compliance with the Internal Revenue Code of 1986, as amended, or the Code. In special, limited circumstances, we have granted stock options, which vested 25% upon grant and 1/36th per month thereafter and expire 10 years from the grant date. Our annual grants to our non-employee directors vest 25% per quarter.

**Restricted Stock and Restricted Stock Units.** Our 2010 Plan authorizes us to grant restricted stock and restricted stock units. We have not issued restricted stock or restricted stock units under the 2010 Plan. The Compensation Committee reviews the relative advantages and disadvantages of restricted stock as a compensation alternative at each annual cycle and may issue restricted stock in the future depending on the analysis in the future.

**Equity Compensation Award**

Following the changes in our fiscal year end from August 31 to December 31, there were no annual stock options granted to our named executive officers during the year ended December 31, 2013. Subsequent to the year ended December 31, 2013, in February 2014, our Board of Directors approved, on the recommendation of the Compensation Committee, to award stock options to our named executive officers, as shown in the table below. All options granted to our named executive officers are intended to be qualified stock options as defined under Section 422 of the Code to the extent possible. The options granted included an adjustment for the 16-month period of service. Dr. Starr did not participate in the discussion or approval of his option grant.

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

**Perquisites and Other Benefits**

Broad-based benefit plans are an integral component of competitive executive compensation packages. Our benefits include a 401(k) savings plan with the Company matching provisions (when such matching is financially viable), healthcare benefits such as medical, dental, and vision plans, and disability and life insurance benefits. We have no structured perquisite benefits, and do not provide any deferred compensation programs or supplemental pensions to any executives. At its discretion, our Compensation Committee may revise, amend or add to the executive's benefits if it deems it advisable.

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

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

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

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## Defined Contribution Plan

We maintain a qualified retirement plan pursuant to Code Sections 401(a) and 401(k) covering substantially all employees, subject to certain minimum age and service requirements, herein referred to as our 401(k) Plan. Our 401(k) Plan allows employees to make voluntary pre-tax contributions. The assets of the 401(k) plan are held in trust for participants and are distributed upon the retirement, disability, death or other termination of employment of the participant.

Employees who participate in our 401(k) Plan may contribute to their 401(k) account up to the maximum amount that varies annually in accordance with the Code. We also make available to 401(k) plan participants the ability to direct the investment of their 401(k) accounts in a well-balanced spectrum of various investment funds.

At our discretion, we provide for a 401(k) Company matching in the amount of 100% of the first 3% of salary that an employee defers and 50% of the next 2% of salary that an employee defers, in compliance with the Internal Revenue Service's Safe Harbor rules.

## Summary Compensation Table

Year Ended December 31, 2013

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

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

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

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

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

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

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

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

This column represents the grant date fair value of the stock options granted during the period to each of our named executive officers, in accordance with ASC Topic 718. For additional information on the valuation assumptions with respect to the four months ended December 31, 2012, please refer to the notes in our

(1) consolidated financial statements included elsewhere in the Form 10-KT. These amounts reflect our accounting expense for these awards, and do not correspond to the actual value, if any, that will be realized by our named executive officers.

With the change in fiscal year from August 31 to December 31 in 2012, the new bonus period covered the (2) September 1, 2012 - December 31, 2013 period. As such, no bonuses were paid during the transition period.

All Other Compensation includes 401(k) matching funded by us, life insurance premiums paid by us where the (3) executive is the beneficiary and employee-taxable commuting benefits.

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

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



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

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

Name and Principal Position	Fiscal Year Ended August 31,	Salary	Option Awards (1)	Non-Equity Incentive Plan Compensation (2)	All Other Compensation (3)	Total
Christopher M. Starr, Ph.D. Chief Executive Officer and Director	2012	\$356,807	\$1,941,447	\$ 112,000	\$ 14,953	\$2,425,207
	2011	346,415	1,232,990	111,996	15,976	1,707,377
Ted Daley President, Raptor Therapeutics (Currently Chief Business Officer)	2012	265,458	639,267	65,000	14,848	984,573
	2011	250,432	406,348	61,839	12,013	730,632

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

consolidated financial statements included elsewhere in the Form 10-KT. These amounts reflect our accounting expense for these awards, and do not correspond to the actual value, if any, that will be realized by our named executive officers.

Cash bonuses for fiscal year 2012 include accruals of bonuses paid in October 2012 based upon milestones achieved by us for the fiscal year ended August 31, 2012. Cash bonuses for fiscal year 2011 include accruals of bonuses paid in September 2011 based upon milestones achieved by us for the fiscal year ended August 31,

(2) 2011.

(3) All Other

Compensation includes 401(k) matching funded by us, life insurance premiums paid by us where the executive is the beneficiary and employee-taxable commuting

benefits.

#### Employment Agreements

Dr. Starr entered into an employment agreement with us in May 2006. Dr. Starr's employment agreement described below is currently still in effect.

Dr. Starr's employment agreement had an initial term of three years commencing on May 1, 2006, and automatically renews for additional one year periods unless either party under such agreement notifies the other that the term will not be extended. Under his agreement, Dr. Starr is entitled to an annual salary of \$150,000, which may be increased from time to time in the discretion of our Board of Directors, and an initial stock option grant to purchase 58,281 shares of our common stock at an exercise price of \$2.83 per share, which vested over three years with a six month cliff vest and expires 10 years from grant date. Dr. Starr's annual salary is subject to annual review and potential increase by our Board of Directors. In addition, he is eligible for annual bonuses based upon our annual bonus compensation program. Information regarding Dr. Starr's annual salary and bonus received during the year ended December 31, 2013 are described in the Compensation Discussion and Analysis section of this annual report. Dr. Starr's employment agreement was amended effective as of January 1, 2009 for purposes of bringing his employment agreement into compliance with the applicable provisions of Section 409A of the Code and the Treasury Regulations and interpretive guidance issued thereunder.

In September 2012, we appointed Georgia L. Erbez as our Chief Financial Officer and, we entered into an employment agreement with Ms. Erbez, or the Erbez Employment Agreement, dated September 10, 2012. The Erbez Employment Agreement has an initial term of three years commencing on September 10, 2012, and renews automatically for successive one year periods, unless either party provides notice to the other terminating the agreement. Under the Erbez Employment Agreement, Ms. Erbez is entitled to an annual salary of \$330,000, the amount of which may be increased from time to time in the discretion of our Board of Directors, and an initial stock option grant to purchase 190,000 shares of our common stock at the closing price on September 7, 2012, the business day preceding the date of grant. These stock options vest 6/48ths on the six-month anniversary of such grant and 1/48th per month thereafter and expire ten years from date of grant. In addition, Ms. Erbez is eligible for annual and discretionary cash bonuses as determined by our Board of Directors, provided, however, that Ms. Erbez must be employed on the date any such bonus actually is paid in order to be eligible to receive such bonus. The annual discretionary bonus has a target payment of 40% of Ms. Erbez's base salary for the year in question.

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On September 10, 2012, we entered into an employment agreement with Julie A. Smith naming her its Executive Vice President, Strategy, and Chief Operating Officer. The agreement provides for similar terms as the Erbez Employment Agreement, except for the following terms. Under the agreement, Ms. Smith is entitled to an annual salary of \$350,000, the amount of which may be increased from time to time in the discretion of our Board of Directors, and an initial stock option grant to purchase 190,000 shares of our common stock at the closing price on September 7, 2012, the business day preceding the date of grant. Further, Raptor will reimburse Ms. Smith for reasonable relocation expenses, not to exceed in the aggregate \$50,000, provided that if her employment with the Company terminates, other than in connection with a "constructive termination" or "termination without cause" (each, as defined in her employment agreement), prior to the second anniversary of her first day of employment with the Company, she will promptly reimburse the Company for the full amount of payment received for such relocation expenses. Ms. Smith was also entitled to reimbursement of commuting expenses related to the performance of Ms. Smith's duties until the earlier of August 31, 2013 or the date Ms. Smith moves her primary residence to the San Francisco Bay Area.

On September 7, 2007, we entered into an employment agreement with Ted Daley for a term of 18 months which automatically renews for additional one year periods unless either party under such agreement notifies the other that the term will not be extended. Under Mr. Daley's agreement, Mr. Daley is entitled to an annual salary of \$150,000 and an initial stock option grant to purchase 34,969 shares of our common stock at an exercise price of \$2.23 per share, which vest over four years with a six month cliff vest and expire 10 years from grant date. In August 2008, the compensation committee recommended, and its Board of Directors approved, a stock option grant to Mr. Daley for the purchase of 23,313 shares of our common stock at an exercise price of \$1.88 per share, which vests 6/48ths upon the six-month anniversary of the grant date and 1/48th per month thereafter and expires ten years from the grant date. Mr. Daley's 2008 stock options were granted in order to increase his initial employment stock option grant to be equal to the stock option grants of our other executive officers. Mr. Daley's annual salary is subject to annual review and potential increase by our Board of Directors. Pursuant to Mr. Daley's employment agreement, Mr. Daley is eligible to receive certain cash bonuses based on triggering events related to the successful development of our Convivia™ product development program. In addition, Mr. Daley is eligible for annual bonuses based upon our annual bonus compensation program. Information regarding Mr. Daley's annual salary and bonuses received during the year ended August 31, 2012 are described below under the headings "Annual Incentive Cash Bonuses" and "Equity Incentive Programs (Currently Based on Stock Options)" above. Mr. Daley's employment agreement was amended effective as of January 1, 2009 for purposes of bringing his employment agreement into compliance with the applicable provisions of Section 409A of the Code and the Treasury Regulations and interpretive guidance issued thereunder.

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

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

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

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If Ms. Erbez's or Ms. Smith's employment is constructively terminated or terminated by us without cause other than during the 12 months following a Change in Control (as defined in the applicable employment agreement), they will be entitled to receive (i) continued payment of base salary for 12 months after such termination; (ii) payment or reimbursement of health plan coverage under COBRA for up to twelve months; (iii) continued exercisability of all of their vested options or stock appreciation rights with respect to our common stock until the first anniversary of the termination of their employment; and (iii) the release from any and all resale or repurchase rights restrictions of all shares of our common stock owned by them. If Ms. Erbez or Ms. Smith is terminated without cause or is constructively terminated by us within the 12 months following a change in control, in addition to the payments described in the preceding sentence, all of their unvested equity and equity-based awards (including stock options) will vest immediately and will remain exercisable until the second anniversary of the termination of employment.

Additionally, they will be entitled to a lump sum payment equal to (i) the average of the annual bonus payments received by them in the two years preceding the year of termination; or (ii) if two annual bonus payment dates have not occurred (regardless of whether they received any annual bonus on such dates) prior to termination, the annual bonus they received with respect to the year preceding the year of termination; or (iii) if an annual bonus payment date has not occurred prior to their termination of employment, 40% of their base salary. If Ms. Erbez or Ms. Smith is terminated for disability, they will continue to receive their base salary and reimbursement or payment of health plan coverage under COBRA for three months following the date of termination. Each of the foregoing payments and benefits is subject to the applicable officer's execution of a general release of claims that becomes effective and irrevocable within 60 days following termination.

If any officer's employment is terminated for cause, by death or due to a voluntary termination, we shall pay to such officer, or in the case of termination due to death, his or her estate, the compensation and benefits payable through the date of termination or, in the case of Ms. Erbez or Ms. Smith, if such officer's employment is terminated by death, a lump sum amount equal to three months of base salary.

## Grants of Plan-Based Awards Table

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

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

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

Table of ContentsOutstanding Equity Awards  
Year Ended December 31, 2013

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

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

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

1/48th per month  
thereafter.

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Options Exercised

Year ended December 31, 2013

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

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

(1) The value realized upon exercise of stock options reflects the price at which shares acquired upon exercise of the stock options were sold or valued for income tax purposes, net of the exercise price for acquiring the shares.

(2) The transactions for each of Dr. Starr and Mr. Daley were made pursuant to a Rule 10b5-1 trading plan adopted by the reporting person.

Executive Payments Upon Termination

Change in control arrangements are designed to retain executives and provide continuity of management in the event of a change in control. These agreements are described in more detail elsewhere in this annual report under the section titled "Employment Agreements" above.

Year Ended December 31, 2013

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

Christopher M. Starr, Ph.D.

Chief Executive Officer and Director

Executive Benefits and Payments Upon Termination	Disability	Death	Termination Without Cause or Constructive Termination	CIC Termination Without Cause or Constructive Termination (1)
Base Salary	\$102,500	(2) \$102,500	\$410,000	(3) \$410,000 (3)
Short-Term Incentive	62,500	(4) -	250,000	(5) 250,000 (5)
Value of Unvested Equity Awards Vesting Continuation/Acceleration	542,973	(6) -	-	2,689,781 (7)
Total	\$707,973	\$102,500	\$660,000	\$3,349,781

(1) "CIC" means change in control, as defined in the officer's employment agreement.

(2) 3 months base salary.

(3) 12 months base salary.

(4) Annual cash bonus pro rata for three months.

(5) Annual cash bonus, payable upon the expiration of the twelve month period following termination.

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

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

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Georgia Erbez

Chief Financial Officer, Secretary and Treasurer

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

(1) "CIC" means change in control, as defined in the officer's employment agreement.

(2) 3 months base salary.

(3) 12 months base salary.

(4) 40% of base salary.

(5) 3 months COBRA continuation.

(6) 12 months COBRA continuation.

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

Julie Anne Smith

EVP, Strategy, Chief Operations Officer

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

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

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Ted Daley  
Chief Business Officer

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

(1) "CIC" means change in control, as defined in the officer's employment agreement.

(2) 3 months base salary.

(3) 6 months base salary.

(4) The target bonus that otherwise would have been earned within the three months following termination.

(5) 50% of the actual bonus earned for the prior fiscal year.

(6) 6 months COBRA continuation.

(7) 3 months continued vesting of the stock options granted under his employment agreement.

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

## Director Compensation

Year ended December 31, 2013

Effective July 16, 2013, non-employee members of our Board of Directors received the following cash compensation:

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

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

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

(2)Mr. Anderson had 394,619 options outstanding as of December 31, 2013, of which 349,617 were exercisable.

(3)Dr. Bruhn had 218,960 options outstanding as of December 31, 2013, of which 154,583 were exercisable.

(4)Dr. Franklin had 318,539 options outstanding as of December 31, 2013, of which 273,537 were exercisable.

(5)Dr. Keltner had 324,164 options outstanding as of December 31, 2013, of which 279,162 were exercisable.

(6)Mr. Sager had 495,531 options outstanding as of December 31, 2013, of which 450,529 were exercisable.

(7)Mr. Samant had 220,000 options outstanding as of December 31, 2013, of which 155,623 were exercisable.

(8)Mr. Walbert had 218,959 options outstanding as of December 31, 2013, of which 154,582 were exercisable.

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

Compensation Committee Interlocks and Insider Participation

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

Compensation Committee or our Board of Directors.

**Compensation Risks**

We believe that risks arising from our compensation policies and practices for our employees are not reasonably likely to have a material adverse effect on the Company. In addition, the Compensation Committee believes that the mix and design of the elements of executive compensation do not encourage management to assume excessive risks.

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The Compensation Committee reviewed the elements of executive compensation to determine whether any portion of executive compensation encouraged excessive risk taking and concluded:

significant weighting towards long-term equity compensation (with multiple year vesting schedules and long expiration terms) discourages short-term risk taking;

for key decision-making officers, base salary makes up a significant majority of cash compensation even with full achievement of annual incentive (cash) awards;

goals are appropriately set to avoid targets that, if not achieved, result in a large percentage loss of compensation;

as a pharmaceutical product development company with industry standard long development timelines, prior to U.S. drug approval in April 2013, we did not face the same level of short-term risks associated with compensation for employees at other companies in rapidly changing markets; the short-term risks in the capital markets faced by us were largely in the approval of and related market exclusivity actions for our product by the FDA and were not under the direct control of our management; and

with a U.S. drug approved in April 2013, as a company with an ultra-orphan product in the critical phase of market launch, the capital market risk has increased due to the short term focus on the emerging product performance in the pharmaceutical marketplace. This potentially affects both short-term cash and long-term equity compensation. Furthermore, compensation decisions include subjective considerations, which moderate the influence of formulaic or objective factors which may encourage excessive risk taking.

Compensation Committee Report

The Compensation Committee has reviewed and discussed the Compensation Discussion and Analysis required by Item 402(b) of Regulation S-K of the SEC's rules and regulations with management and, based on such review and discussions, the Compensation Committee recommended to the Board of Directors that the Compensation Discussion and Analysis be included in our Annual Report on Form 10-K and proxy statement for the 2014 annual meeting of stockholders.

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

This foregoing compensation committee report is not "soliciting material," is not deemed "filed" with the SEC, and shall not be deemed incorporated by reference by any general statement incorporating by reference this Annual Report on Form 10-K into any filing of ours under the Securities Act of 1933, as amended, or under the Exchange Act, except to the extent we specifically incorporate this report by reference.

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## ITEM 12: SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

## Equity Compensation Plan Information

The following table provides certain information with respect to all of our equity compensation plans in effect as of December 31, 2013:

Plan Category	Number of securities to be issued upon exercise of outstanding options, warrants and rights	Weighted average price of outstanding options, warrants and rights	Number of securities remaining available for future issuance under equity compensation plans
Equity compensation plans approved by stockholders	8,217,674	\$ 5.77	3,861,729
Equity compensation plans not approved by stockholders	—	—	—
Total	8,217,674	\$ 5.77	3,861,729

## Security Ownership of Certain Beneficial Owners, Directors and Management

The following table sets forth, as of February 28, 2014, any person or group known by us to be the beneficial owner of more than five percent (5%) of any class of our voting securities, each of our named executive officers, each of our directors and all of our current executive officers and directors as a group. Except as otherwise indicated, each listed stockholder directly owned his or her shares and had sole voting and investment power. Unless otherwise noted, the address for each person listed below is Raptor Pharmaceutical Corp., 5 Hamilton Landing, Suite 160, Novato, CA 94949.

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

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

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\* Less than one percent.

Beneficial ownership is determined in accordance with SEC rules and generally includes voting or investment power with respect to securities.

Shares of common stock subject to stock options, warrants and convertible preferred stock that may be

(1) acquired within sixty (60) days of February 28, 2014 are counted as outstanding for computing the percentage held by each person holding such options or warrants but are not counted as outstanding for computing the percentage of any other person.

The shares reported in this column represent shares of common stock underlying stock options, warrants and

(2) convertible preferred stock exercisable or convertible for shares of our common stock within sixty (60) days of February 28, 2014.

Based on 62,479,286

(3) shares outstanding as of February 28, 2014.

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

(4) The principal business address for BlackRock, Inc. is 40 East 52<sup>nd</sup>

Street, New York, NY  
10022.

The principal business  
address for Credit Suisse

(6) AG is Uetlibergstrasse  
231, CH 8070, Zurich,  
Switzerland.

The principal business  
address for FMR LLC is

(7) 245 Summer Street,  
Boston, MA 02210.

Includes 699,370 shares  
our common

stock owned by the  
Christopher M. and S.L.  
Starr Trust of which Dr.  
Starr is a co-trustee and

(8) beneficiary and shares  
voting and investment  
power, and options to  
purchase 767,660 shares  
of our common stock  
held by Dr. Starr  
directly as of February  
28, 2014.

Includes 3,500 shares  
our common stock held  
in trust for Ms. Erbez's  
children of which Ms.  
Erbez is a co-trustee and  
beneficiary and shares

(9) voting and investment  
power, and options to  
purchase 75,207 shares  
of our common stock  
held by Ms. Erbez  
directly as of February  
28, 2014.

#### ITEM 13: CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE Transactions with Related Persons

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

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

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

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

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

Since January 1, 2013, there has not been nor is there currently proposed any transaction or series of similar transactions to which we were or are to be a party in which the amount involved exceeds \$120,000 and in which any of our directors, executive officers, nominees for director, persons who we know hold more than 5% of our common stock, or any member of the immediate family of any of the foregoing persons had or will have a direct or indirect material interest other than: (i) compensation agreements and other arrangements, which are described elsewhere in this annual report, and (ii) the transactions described below.

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We have entered into indemnity agreements with all of our officers and directors which provide, among other things, that we will indemnify such officer or director, under the circumstances and to the extent provided for therein, for expenses, damages, judgments, fines and settlements he or she may be required to pay in actions or proceedings which he or she is or may be made a party by reason of his or her position as a director, officer or other agent of us or our subsidiaries, and otherwise to the fullest extent permitted under the General Corporation Law of the State of Delaware and our Bylaws.

**Indebtedness of Directors and Executive Officers**

None of our directors or executive officers or associates of any director or executive officer is or at any time since September 1, 2011 has been indebted to us.

**Independence of Our Board of Directors**

Our Board of Directors has determined that as of December 31, 2013, all current members of our Board of Directors are independent (as independence is currently defined in Rule 5605(a)(2) of the NASDAQ listing standards), except for Dr. Starr. Our Board of Directors has also determined that each member of our Audit Committee, Compensation Committee and Corporate Governance and Nominating Committee is independent as defined by the SEC and NASDAQ rules.

**ITEM 14: PRINCIPAL ACCOUNTING FEES AND SERVICES**

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

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

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

Description of Services Provided by BPM	Year ended December 31, 2013*	Four months ended December 31, 2012	Year ended August 31,	
			2012	2011
Audit Fees: These services relate to the audit and review of financial statements, assurance and other related services.	\$261,237	\$194,480	\$386,282	\$304,382
Tax Compliance Fees: These services relate to the preparation of federal, state and foreign tax returns and other filings.	86,060	0	38,631	28,839
Tax Consulting and Advisory Services: These services primarily relate to the area of tax strategy and minimizing Federal, state, local and foreign taxes.	5,252	68,726	259,113	0

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

As provided in the Audit Committee charter, the Audit Committee pre-approves all of the services provided by our independent registered public accounting firm. 100% of the above services and estimates of the expected fees were reviewed and approved by the Audit Committee before the respective services were rendered.

The Audit Committee has considered the nature and amount of the fees billed by Grant Thornton and believes that the provision of the services for activities is compatible with maintaining Grant Thornton's independence.

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PART IV

ITEM 15: EXHIBITS, FINANCIAL STATEMENT SCHEDULES

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

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

1) Index list to Consolidated Financial Statements:

	Page
Reports of Independent Registered Public Accounting Firm	91
Reports of Former Independent Registered Public Accounting Firm	93
Consolidated Balance Sheets as of December 31, 2013 and December 31, 2012	94
Consolidated Statements of Operations and Comprehensive Loss for the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal years ended August 31, 2012 and 2011	95
Consolidated Statements of Stockholders' Equity (Deficit) for the fiscal years ended August 31, 2011 and 2012, the four months ended December 31, 2012 and the year ended December 31, 2013	96
Consolidated Statements of Cash Flows for the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal years ended August 31, 2012 and 2011	98
Notes to Consolidated Financial Statements	100

2) Schedule II is included on page 121 of this report. All other schedules are omitted because they are not required or the required information is included in the financial statements or notes thereto.

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Exhibits

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

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

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

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

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

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10.33++	API Supply Agreement, dated November 15, 2010, between Cambrex Profarmaco Milano and Raptor Therapeutics Inc.	POS AM11/23/2010	10.54	
10.34++	Cooperative Research and Development Agreement for Extramural-PHS Clinical Research, dated December 15, 2011, between the U.S. Department of Health and Human Services, as represented by the National Institute of Diabetes and Digestive and Kidney Diseases, an institute or center of the National Institutes of Health, and Raptor Therapeutics Inc.	10-Q	4/9/2012	10.1
10.36	Sales Agreement, dated April 30, 2012, between Cowen and Company, LLC and the Registrant.	8-K	5/1/2012	1.1
10.37++	Second Amendment to License Agreement, effective October 30, 2012, between The Regents of the University of California and Raptor Therapeutics, Inc.	10-KT	3/14/2013	10.37
10.38++	Loan Agreement, dated December 20, 2012, among HealthCare Royalty Partners II, L.P., the Guarantors party thereto and the Registrant.	10-KT/A	6/19/2013	10.1
10.39++	Wholesale Product Purchase Agreement, dated April 3, 2013, between Accredo Health Group, Inc. and Raptor Pharmaceuticals Inc.	10-Q	8/9/2013	10.1
10.40++	Pharmacy Services Agreement, dated April 3, 2013, between Accredo Health Group, Inc. and Raptor Pharmaceuticals Inc.	10-Q	8/9/2013	10.2
10.41	Office Lease, dated April 18, 2013, between Hamilton Marin, LLC and Raptor Pharmaceuticals Corp.	10-Q	8/9/2013	10.3
10.42	First Amendment to Lease, dated June 10, 2013, between Hamilton Marin, LLC and Raptor Pharmaceuticals Corp.	10-Q	8/9/2013	10.4
10.43++	Amendment to Manufacturing Services Agreement, dated April 5, 2012, between Patheon Pharmaceuticals Inc. and Raptor Therapeutics, Inc.	10-Q	8/9/2013	10.5
10.44++	Second Amendment to Manufacturing Services Agreement, dated June 21, 2013, between Patheon Pharmaceuticals Inc. and Raptor Therapeutics, Inc.	10-Q	8/9/2013	10.6
21.1	Subsidiaries of the Registrant.			X
23.1	Consent of Grant Thornton LLP, Independent Registered Public Accounting Firm to the Registrant			X
23.2	Consent of Burr Pilger Mayer, Inc., Former Independent Registered Public Accounting Firm to the Registrant			X
24.1	Power of Attorney (included in the signature page hereto).			X
31.1	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director.			X
31.2	Certification of Georgia Erbez, Chief Financial Officer, Secretary and Treasurer.			X
32.1	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director, and of Georgia Erbez, Chief Financial Officer, Secretary and Treasurer.			X
*	The Raptor Pharmaceuticals Corp. warrants set forth in Exhibits 4.6 - 4.9 have been converted into warrants of the Registrant, and the exercise price of such warrants and number of shares of common stock issuable thereunder have been converted as described in Item 1.01 (under the section titled, "Background") of the Registrant's Current Report on Form 8-K, filed on October 5, 2009.			
**	Incorporated by reference from the indicated filing of Raptor Pharmaceuticals Corp. rather than that of the Registrant.			
#	Indicates a management contract or compensatory plan or arrangement.			
+	Certain information omitted pursuant to a request for confidential treatment filed with the SEC.			
++	Certain information omitted pursuant to a request for confidential treatment filed separately with and granted by the SEC.			



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## SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

RAPTOR PHARMACEUTICAL CORP.

Dated: March 17, 2014

By : /s/ Georgia Erbez

Georgia Erbez

Chief Financial Officer, Secretary and Treasurer

(Principal Financial Officer and Principal Accounting Officer)

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Christopher M. Starr, Ph.D. and Georgia Erbez, his or her attorney-in-fact, with the power of substitution, for him or her in any and all capacities, to sign any amendments to this Annual Report on Form 10-K and to file the same, with exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, hereby ratifying and confirming all that each of said attorneys-in-fact, or his or her substitute or substitutes, may do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated:

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

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Erich Sager

Director

/s/ Vijay B. Samant

March 17, 2014

Vijay B. Samant

Director

/s/ Timothy P. Walbert

March 17, 2014

Timothy P. Walbert

Director

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Explanatory Note

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

Financial Statements

The following consolidated financial statements of Raptor Pharmaceutical Corp. and the Independent Registered Public Accounting Firm's Report issued thereon, are incorporated by reference in Part II, Item 8 of this Annual Report on Form 10-K:

	Page
<u>Reports of Independent Registered Public Accounting Firm</u>	91
Report of the Company's <u>Former Independent Registered Public Accounting Firm</u>	93
<u>Consolidated Balance Sheets as of December 31, 2013 and December 31, 2012</u>	94
<u>Consolidated Statements of Operations and Comprehensive Loss for the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal years ended August 31, 2012 and 2011</u>	95
<u>Consolidated Statements of Stockholders' Equity (Deficit) for the fiscal years ended August 31, 2011 and 2012, the four months ended December 31, 2012 and the year ended December 31, 2013</u>	96
<u>Consolidated Statements of Cash Flows for the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal years ended August 31, 2012 and 2011</u>	98
<u>Notes to Consolidated Financial Statements</u>	100

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

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

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

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

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of Raptor Pharmaceutical Corp. and subsidiaries as of December 31, 2013 and 2012, and the results of their operations and their cash flows for the year ended December 31, 2013 and for the four month period ended December 31, 2012 in conformity with accounting principles generally accepted in the United States of America. Also in our opinion, the related financial statement schedule, when considered in relation to the basic consolidated financial statements taken as a whole, presents fairly, in all material respects, the information set forth therein.

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

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

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

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

We have audited the internal control over financial reporting of Raptor Pharmaceutical Corp. (a Delaware corporation) and subsidiaries (the "Company") as of December 31, 2013, based on criteria established in the 1992 Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2013, based on criteria established in the 1992 Internal Control—Integrated Framework issued by COSO.

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

/s/ GRANT THORNTON LLP

San Francisco, California

March 17, 2014

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

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

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

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

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

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

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## Raptor Pharmaceutical Corp.

## Consolidated Balance Sheets

(In thousands, except for per share data)

	December 31,	
	2013	2012
Assets		
Current assets:		
Cash and cash equivalents	\$83,052	\$36,313
Restricted cash	500	163
Short-term investments	0	22,096
Accounts receivable	6,181	0
Inventories	3,000	0
Prepaid expenses and other	3,566	1,610
Total current assets	96,299	60,182
Intangible assets, net	3,213	2,156
Goodwill	3,275	3,275
Fixed assets, net	1,810	416
Other assets	4,129	2,094
Total assets	\$108,726	\$68,123
Liabilities and Stockholders' Equity (Deficit)		
Liabilities		
Current liabilities:		
Accounts payable	\$5,264	\$4,599
Accrued liabilities	12,767	2,150
Deferred revenue	4,698	0
Common stock warrant liability	7,066	16,405
Deferred rent	302	6
Capital lease liability - current	18	8
Total current liabilities	30,115	23,168
Note payable	50,000	25,000
Capital lease liability - long-term	41	11
Total liabilities	80,156	48,179
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.001 par value, 15,000 shares authorized, zero shares issued and outstanding	0	0
Common stock, \$0.001 par value, 150,000 shares authorized, 61,615 and 52,425 shares issued and outstanding at December 31, 2013 and December 31, 2012, respectively	62	52

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

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

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

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

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

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

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

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

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## Raptor Pharmaceutical Corp.

## Consolidated Statements of Stockholders' Equity (Deficit) (Continued)

(In thousands, except for per share data)

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

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

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Raptor Pharmaceutical Corp.  
 Consolidated Statements of Cash Flows  
 (In thousands, except for per share data)

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

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

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Supplemental cash flow information:

Interest paid	\$5,142	\$83	\$3	\$2
Income taxes paid	\$2	\$0	\$0	\$0

Supplemental disclosure of non-cash financing activities:

Common stock issued as fee for equity line	\$0	\$0	\$0	\$519
Acquisition of equipment in exchange for capital lease	\$60	\$0	\$13	\$14
Fair value of warrant liability reclassified to equity upon exercise	\$20,086	\$2,345	\$9,482	\$8,506

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

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RAPTOR PHARMACEUTICAL CORP.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands, except for per share data, or unless otherwise specified)

(1) NATURE OF OPERATIONS AND BUSINESS RISKS

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

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

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

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

Change in Fiscal Year End

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

Basis of Presentation

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

accounts have been eliminated.

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RAPTOR PHARMACEUTICAL CORP.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands, except for per share data, or unless otherwise specified)

(2) SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Use of Estimates

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

Functional Currency

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

Revenue Recognition and Accounts Receivable

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

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

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

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

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Inventories and Cost of Sales

Inventories are stated at the lower of cost or market price, with cost determined on a first-in, first-out basis.

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

Fair Value of Financial Instruments

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

Cash and Cash Equivalents

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

Short-term Investments

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

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Prepaid Expenses and Other

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

Deferred Offering Costs

Deferred offering costs represent expenses incurred to raise equity capital related to financing transactions which have not yet been completed as of the balance sheet dates.

Note Payable and Debt Issuance Costs

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

Intangible Assets

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

Fixed Assets

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

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RAPTOR PHARMACEUTICAL CORP.

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Segment Information

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

Impairment of Goodwill and Intangible Assets

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

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

Common Stock Warrant Liabilities

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

Research and Development

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

Advertising Expenses

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

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## RAPTOR PHARMACEUTICAL CORP.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands, except for per share data, or unless otherwise specified)

## Net Loss per Share

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

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

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

## Comprehensive Loss

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

## Income Taxes

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

The Company identifies uncertain tax positions and discloses any potential tax liability on their financial statements.

The Company recognizes interest and/or penalties related to income tax matters as a component of income tax expense. As of December 31, 2013, there was no accrued interest and penalties related to uncertain tax positions.

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

## Stock Option Plan

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

## Reclassifications

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

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## RAPTOR PHARMACEUTICAL CORP.

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(In thousands, except for per share data, or unless otherwise specified)

## (3) INTANGIBLE ASSETS AND GOODWILL

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

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

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

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

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

A summary of intangibles acquired is as follows:

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

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

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

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

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## RAPTOR PHARMACEUTICAL CORP.

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(In thousands, except for per share data, or unless otherwise specified)

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

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

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

## (4) FIXED ASSETS

Fixed assets consisted of:

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

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

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## RAPTOR PHARMACEUTICAL CORP.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands, except for per share data, or unless otherwise specified)

## (5) FAIR VALUE MEASUREMENT

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

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

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

	Level			December 31, 2013
	Level 1	2	Level 3	
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

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

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## RAPTOR PHARMACEUTICAL CORP.

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(In thousands, except for per share data, or unless otherwise specified)

Cash equivalents and short-term investments represent the fair value of the Company's investments in money market funds and a short-term bond fund as of December 31, 2013 and December 31, 2012.

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

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

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

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

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## RAPTOR PHARMACEUTICAL CORP.

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(In thousands, except for per share data, or unless otherwise specified)

## (6) NOTE PAYABLE

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

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

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

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

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

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## RAPTOR PHARMACEUTICAL CORP.

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(In thousands, except for per share data, or unless otherwise specified)

## (7) ACCRUED LIABILITIES

Accrued liabilities consisted of:

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

## (8) STOCK OPTION PLANS

## 2010 Stock Incentive Plan

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

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## RAPTOR PHARMACEUTICAL CORP.

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(In thousands, except for per share data, or unless otherwise specified)

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

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

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

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

\*Dividend rate is 0% for all periods presented.

The compensation expense for stock-based compensation awards includes an estimate for forfeitures and is recognized over the requisite service period of the options, which is typically the period over which the options vest, using the straight-line method.

The Company also recognizes as an expense the fair value of options granted to persons who are neither employees nor directors. The fair value of expensed options was based on the Black-Scholes option-pricing model assuming the same factors shown in the stock-based compensation expense table above. Stock-based compensation expense for consultants for the year ended December 31, 2013, the four months ended December 31, 2012 and the years ended August 31, 2012 and 2011, was \$4, \$9, \$72 and \$197, respectively.

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## RAPTOR PHARMACEUTICAL CORP.

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(In thousands, except for per share data, or unless otherwise specified)

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

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

The number of options outstanding, vested and expected to vest as of December 31, 2013 was 8,109 and the weighted-average remaining contractual life was 7.6 years. The aggregate intrinsic value and the weighted-average intrinsic value of stock options outstanding, vested and expected to vest as of December 31, 2013 was \$71.8 million and \$8.85 per option, respectively. The number of options outstanding, vested and expected to vest as of December 31, 2012 was 7,644 and the weighted-average remaining contractual life was 8.21 years. The aggregate intrinsic value and the weighted-average intrinsic value of stock options outstanding, vested and expected to vest as of December 31, 2012 was \$10.3 million and \$1.34 per option, respectively.

As of December 31, 2013, the options outstanding under all of the Company's stock option plans consisted of the following (in thousands, except per share data):

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

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

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

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

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## RAPTOR PHARMACEUTICAL CORP.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands, except for per share data, or unless otherwise specified)

## (9) INCOME TAXES

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

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

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

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

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

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

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands, except for per share data, or unless otherwise specified)

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

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

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

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

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

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

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

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

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

(10) ISSUANCE OF COMMON STOCK

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

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

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

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

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RAPTOR PHARMACEUTICAL CORP.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands, except for per share data, or unless otherwise specified)

2009 MERGER AND NASDAQ LISTING

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

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

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

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

ISSUANCES OF COMMON STOCK IN CONNECTION WITH AN EQUITY LINE

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

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

2010 PRIVATE PLACEMENT

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

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RAPTOR PHARMACEUTICAL CORP.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands, except for per share data, or unless otherwise specified)

2011 FOLLOW-ON PUBLIC OFFERING

On September 13, 2011, the Company closed an underwritten public offering of shares of the Company's common stock at a price to the public of \$4.00 per share. The shares sold in the offering included 10.0 million shares of common stock plus an additional 1.5 million shares of common stock pursuant to the exercise by the underwriters of the over-allotment option the Company granted to them. Total gross proceeds to the Company in the offering (including in connection with the sale of the shares of common stock pursuant to the exercise of the over-allotment option) totaled \$46.0 million, before underwriting discounts and commissions. The offering resulted in net proceeds to the Company of approximately \$42.8 million after deduction of underwriting discounts of 6% and other offering expenses paid by the Company.

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

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

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

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

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## RAPTOR PHARMACEUTICAL CORP.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands, except for per share data, or unless otherwise specified)

## (11) WARRANTS

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

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

## \*Weighted-average exercise price

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

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

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

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



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## RAPTOR PHARMACEUTICAL CORP.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands, except for per share data, or unless otherwise specified)

## (12) COMMITMENTS AND CONTINGENCIES

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

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

## LEASES

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

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

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

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

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



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## RAPTOR PHARMACEUTICAL CORP.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands, except for per share data, or unless otherwise specified)

## (13) QUALIFYING THERAPEUTIC DISCOVERY PROJECT GRANT

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

## (14) QUARTERLY RESULTS OF OPERATIONS (UNAUDITED)

The following table presents selected unaudited quarterly results of operations in conjunction with the consolidated financial statements and related notes contained elsewhere in this Annual Report on Form 10-K. These unaudited results were prepared on the same basis as the Company's audited consolidated financial statements. The Company's quarterly operating results have fluctuated in the past and may continue to do so in the future as a result of a number of factors, including, but not limited to, the timing and amounts of its revenues and the timing and nature of research and development activities.

	(In millions, except per share data, unaudited)			
	March			
Quarterly Data 2013:	31, 2013	June 30, 2013	September 30, 2013	December 31, 2013
Net sales	\$0	\$0	\$ 6.7	\$ 10.2
Net loss	\$(15.9)	\$(24.1 )	\$( 17.3 )	\$( 12.1 )
Net loss per share, basic and diluted	\$(0.30)	\$(0.43 )	\$( 0.29 )	\$( 0.20 )
	November 30, 2012			
Quarterly Data for the Four Months Ended December 31, 2012:	(1)			
Net loss	\$(13.4)			
Net loss per share, basic and diluted	\$(0.26)			
	November 30, 2011			
Quarterly Data 2012:	February 29, 2012	May 31, 2012	August 31, 2012	
Net loss	\$(11.4)	\$(14.0 )	\$( 3.0 )	\$( 10.2 )
Net loss per share, basic and diluted	\$(0.25)	\$(0.29 )	\$( 0.06 )	\$( 0.21 )

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Quarterly Data 2011:	November			
	30, 2010	February 28, 2011	May 31, 2011	August 31, 2011
Net loss	\$(10.1)	\$(3.0 )	\$(20.3 )	\$(3.8 )
Net loss per share, basic and diluted	\$(0.33)	\$(0.09 )	\$(0.62 )	\$(0.11 )

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

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RAPTOR PHARMACEUTICAL CORP.

Schedule II: Valuation and Qualifying Accounts  
(in millions)

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

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