

LIGAND PHARMACEUTICALS INC

Form 10-K/A

December 12, 2014

Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

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FORM 10-K/A

Amendment No. 2

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Mark One

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

For the Fiscal Year Ended December 31, 2013

OR

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_.

Commission File No. 001-33093

LIGAND PHARMACEUTICALS INCORPORATED

(Exact name of registrant as specified in its charter)

Delaware

77-0160744

(State or other jurisdiction of  
incorporation or organization)

(IRS Employer  
Identification No.)

11119 North Torrey Pines Rd., Suite 200  
La Jolla, CA

92037

(Address of Principal Executive Offices)

(Zip Code)

Registrant's telephone number, including area code: (858) 550-7500

Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class

Name of Each Exchange on Which Registered

Common Stock, par value \$.001 per share

The NASDAQ Global Market of The NASDAQ Stock Market LLC

Preferred Share Purchase Rights

The NASDAQ Global Market of The NASDAQ Stock Market LLC

Securities registered pursuant to Section 12(g) of the Act:

None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☒ No ☐

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Securities Exchange Act of 1934. Yes ☐ No ☒

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

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements

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incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☒

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

Large Accelerated Filer ☐ Accelerated Filer ☒ Non-accelerated Filer ☐ Smaller reporting company ☐  
(Do not check if a smaller reporting company)

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

The aggregate market value of the Registrant's voting and non-voting stock held by non-affiliates was approximately \$676.8 million based on the last sales price of the Registrant's Common Stock on the NASDAQ Global Market of the NASDAQ Stock Market LLC on June 30, 2013. For purposes of this calculation, shares of Common Stock held by directors, officers and 10% stockholders known to the Registrant have been deemed to be owned by affiliates which should not be construed to indicate that any such person possesses the power, direct or indirect, to direct or cause the direction of the management or policies of the Registrant or that such person is controlled by or under common control with the Registrant.

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Table of Contents

As of December 12, 2014, the Registrant had 19,645,775 shares of Common Stock outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the Proxy Statement for the Registrant's 2014 Annual Meeting of Stockholders filed with the Commission on April 21, 2014 are incorporated by reference in Part III of this Annual Report on Form 10-K. With the exception of those portions that are specifically incorporated by reference in this Annual Report on Form 10-K, such Proxy Statement shall not be deemed filed as part of this Report or incorporated by reference herein.

EXPLANATORY NOTE

Ligand Pharmaceuticals Incorporated ("the Company") is filing this Amendment No. 2 (this "Amendment") to its Annual Report on Form 10-K for the fiscal year ended December 31, 2013, originally filed with the Securities and Exchange Commission (the "SEC") on February 24, 2014 (the "Original Filing Date") and amended on Form 10-K/A (the "Amended Form 10-K/A") on December 1, 2014 (the "Amended Filing Date"), solely for the purpose of correcting coding errors in certain dates included in the XBRL ("eXtensible Business Reporting Language") exhibits filed with the Amended Form 10-K/A and updated references in the consent of independent registered public accounting firm. This Amendment speaks as of the Original Filing Date, has not been revised to reflect events that have occurred subsequent to the Original Filing Date, and does not modify or update in any way any other disclosures made in the original Form 10-K. Consequently, this Amendment should be read in conjunction with the Company's other filings with the SEC made subsequent to the Original Filing Date.

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## Table of Contents

### Table of Contents

#### Part I

Item 1.	<u>Business</u>	<u>1</u>
Item 1A.	<u>Risk Factors</u>	<u>15</u>
Item 1B.	<u>Unresolved Staff Comments</u>	<u>22</u>
Item 2.	<u>Properties</u>	<u>22</u>
Item 3.	<u>Legal Proceedings</u>	<u>23</u>
Item 4.	<u>Mine Safety Disclosures</u>	<u>23</u>

#### Part II

Item 5.	<u>Market for Registrant's Common Equity, Related Stockholder Matters, and Issuer Purchases of Equity Securities</u>	<u>23</u>
Item 6.	<u>Selected Consolidated Financial Data</u>	<u>25</u>
Item 7.	<u>Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	<u>26</u>
Item 7A.	<u>Quantitative and Qualitative Disclosures About Market Risk</u>	<u>39</u>
Item 8.	<u>Consolidated Financial Statements and Supplementary Data</u>	<u>39</u>
Item 9.	<u>Changes in and Disagreements with Accountants on Accounting and Financial Disclosure</u>	<u>73</u>
Item 9A.	<u>Controls and Procedures</u>	<u>74</u>

#### Part III

Item 10.	<u>Directors, Executive Officers and Corporate Governance</u>	<u>77</u>
Item 11.	<u>Executive Compensation</u>	<u>77</u>
Item 12.	<u>Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters</u>	<u>77</u>
Item 13.	<u>Certain Relationships and Related Transactions, and Director Independence</u>	<u>77</u>
Item 14.	<u>Principal Accountant Fees and Services</u>	<u>77</u>

#### Part IV

Item 15.	<u>Exhibits, Financial Statement Schedules</u>	<u>78</u>
	<u>SIGNATURES</u>	<u>86</u>

#### AVAILABLE INFORMATION:

We file electronically with the Securities and Exchange Commission, or the SEC, our annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K and, as necessary, amendments to these reports, pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended. The public may read or copy any materials we file with the SEC at the SEC's Public Reference Room at 100 F Street, NE, Washington, DC 20549. The public may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. The SEC maintains an Internet site that contains reports, proxy and information statements, and other information regarding issuers that file such documents electronically with the SEC. The address of that site is <http://www.sec.gov>.

You may obtain a free copy of our annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K and amendments to those reports which are posted as soon as reasonably practicable after filing on our website at <http://www.ligand.com>, by contacting the Investor Relations Department at our corporate offices by calling (858) 550-7500 or by sending an e-mail message to [investors@ligand.com](mailto:investors@ligand.com). You may also request information via the Investor Relations page of our website.

Table of Contents

PART I

Cautionary Note Regarding Forward-Looking Statements:

You should read the following together with the more detailed information regarding our company, our common stock and our financial statements and notes to those statements appearing elsewhere in this document or incorporated by reference. The SEC allows us to “incorporate by reference” information that we file with the SEC, which means that we can disclose important information to you by referring you to those documents. The information incorporated by reference is considered to be part of this report.

This report and the information incorporated herein by reference contain forward-looking statements that involve a number of risks and uncertainties. Although our forward-looking statements reflect the good faith judgment of our management, these statements can only be based on facts and factors currently known by us. Consequently, these forward-looking statements are inherently subject to risks and uncertainties, and actual results and outcomes may differ materially from results and outcomes discussed in the forward-looking statements.

Forward-looking statements can be identified by the use of forward-looking words such as “believes,” “expects,” “hopes,” “may,” “will,” “plan,” “intends,” “estimates,” “could,” “should,” “would,” “continue,” “seeks,” “pro forma,” or “anticipates,” or words (including their use in the negative), or by discussions of future matters such as those related to our royalty revenues, collaborative revenues and milestones, and product development, as well as other statements that are not historical. These statements include but are not limited to statements under the captions “Business,” “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” as well as other sections in this report. You should be aware that the occurrence of any of the events discussed under the caption “Risk Factors” and elsewhere in this report could substantially harm our business, results of operations and financial condition and that if any of these events occurs, the trading price of our stock could decline and you could lose all or a part of the value of your investment in our stock.

The cautionary statements made in this report are intended to be applicable to all related forward-looking statements wherever they may appear in this report. We urge you not to place undue reliance on these forward-looking statements, which speak only as of the date of this report. Except as required by law, we assume no obligation to update our forward-looking statements, even if new information becomes available in the future. This caution is made under the safe harbor provisions of Section 21E of the Securities Exchange Act of 1934, as amended.

References to “Ligand Pharmaceuticals Incorporated,” “Ligand,” the “Company,” “we,” “our” and “us” include our wholly owned subsidiaries-Ligand JVR, Allergan Ligand Retinoid Therapeutics, Seragen, Inc., Pharmacopeia, LLC, or Pharmacopeia, Neurogen Corporation, or Neurogen, CyDex Pharmaceuticals, Inc., or CyDex, Metabasis Therapeutics, or Metabasis, and Nexus Equity VI LLC.

## Table of Contents

### Item 1. Business

#### Overview

We are a biotechnology company that operates with a business model focused on developing or acquiring revenue generating assets and coupling them with a lean corporate cost structure. Our goal is to create a sustainably profitable business and generate meaningful value for our stockholders. Since a portion of our business model is based on the goal of partnering with other pharmaceutical companies to commercialize and market our assets, a significant amount of our revenue is based largely on payments made to us by partners for royalties, milestones and license fees. We recognized the important role of the drug reformulation segment in the pharmaceutical industry and in 2011 added Captisol® to our technology portfolio. Captisol is a formulation technology that has enabled six FDA approved products, including Onyx's Kyprolis® and Baxter International's Nexterone® and is currently being developed in a number of clinical-stage partner programs. In comparison to our peers, we believe we have assembled one of the largest and most diversified asset portfolios in the industry with the potential to generate significant revenue in the future. The therapies in our development portfolio address the unmet medical needs of patients for a broad spectrum of diseases including hepatitis, immune (idiopathic) thrombocytopenic purpura, or ITP, muscle wasting, multiple myeloma, Alzheimer's disease, dyslipidemia, diabetes, anemia, epilepsy, Focal Segmental Glomerulosclerosis, or FSGS, and osteoporosis. We have established multiple alliances with the world's leading pharmaceutical companies including GlaxoSmithKline, Onyx Pharmaceuticals (a subsidiary of Amgen, Inc.), Merck, Pfizer, Baxter International, Lundbeck Inc., Eli Lilly and Co., and Spectrum Pharmaceuticals, Inc.

We were incorporated in Delaware in 1987. Our principal executive offices are located at 11119 North Torrey Pines Road, Suite 200, La Jolla, California, 92037. Our telephone number is (858) 550-7500.

#### Business Strategy

Our business model is designed to create value for stockholders by assembling a diversified portfolio of biotech and pharmaceutical revenue streams and operating that business with an efficient and low corporate cost structure. Our goal is to become a sustainably profitable company that offers investors an opportunity to participate in the promise of the biotech industry in a diversified, lower-risk business than a typical biotech company. Our business model is based on the concept of doing what we do best: drug discovery, reformulation and partnering with other pharmaceutical companies to leverage what they do best (late-stage development, regulatory management and commercialization) to ultimately generate our revenue. Our revenue consists mostly of license fees, milestones, royalties from the partners that license our drugs and technologies, and Captisol material sales. In addition to discovering our own proprietary drugs, we use an aggressive acquisition strategy to bring in new assets, pipelines, and technologies to aid in generating additional potential new revenue streams. The principal elements of our strategy are set forth below.

We are assembling a large portfolio of fully funded programs through acquisition and licensing to drive future profitability. We have assembled a portfolio of over 90 fully funded partner programs that are in all stages of development, from preclinical research to awaiting commercialization. Fully funded programs are those for which our partners pay all of the development and commercialization costs. These assets represent the next wave of potential marketed drugs that could generate revenue for us. We assemble this portfolio by either licensing out our own proprietary drug development programs or acquiring existing partnered programs from other companies. For our internal programs, we generally plan to advance drug candidates through early-stage drug development and/or clinical proof-of-concept. We believe partnerships are not only a source of research funding, license fees, future milestone payments and royalties, but they also position our assets with companies that have the expertise to obtain regulatory approval and successfully launch and commercialize these assets. We believe that focusing on discovery and early-stage drug development while benefiting from our partners' proven development and commercialization expertise will reduce our internal expenses and allow us to have a larger number of drug candidates progress to later stages of drug development.

We sell Captisol material to a broad range of customers. We are the sole provider of a proprietary formulation technology known as Captisol. Captisol is a well validated chemically-modified cyclodextrin that improves the solubility, stability, and pharmacokinetics of many drugs. We generate revenue by selling Captisol material to our partners that have either licensed our proprietary Captisol-enabled drugs or have taken a license to use Captisol with their own internal programs.

We discover and develop compounds that are promising drug candidates. We discover, synthesize and test numerous compounds to identify those that are most promising for clinical development. We perform extensive target profiling and base our selection of promising development candidates on product characteristics such as initial indications of safety and efficacy. We believe that this focused strategy allows us to eliminate unpromising candidates

## Table of Contents

from consideration sooner without incurring substantial clinical costs. Our goal is to partner our programs early in the development and regulatory life-cycle.

### Our Asset Portfolio

We have a large portfolio of current and future potential revenue-generating programs, over 90 of which are fully-funded by our partners. Over 70% of our 2013 revenue is derived from our Promacta®, Kyprolis, and Captisol programs (including Captisol material sales).

### Material Late-Stage Development or Commercial Programs

We have multiple partnered programs in our portfolio that are either in or nearing the regulatory approval process. These programs represent the next series of potential royalty generating assets in our portfolio.

#### Promacta (GSK)

GSK's Promacta (Eltrombopag) is the first oral thrombopoietin (TPO) receptor agonist therapy for the treatment of adult patients with chronic immune (idiopathic) thrombocytopenic purpura, or ITP. In late 2008, the U.S. Food and Drug Administration, or FDA, granted accelerated approval of Promacta for the treatment of thrombocytopenia in patients with chronic ITP, who have had an insufficient response to corticosteroids, immunoglobulins or splenectomy. In 2010, GSK received approval for Revolade® (eltrombopag/Promacta) from the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) and from the Japanese Ministry of Health, Labour and Welfare for the oral treatment of thrombocytopenia (reduced platelet count) in adults with the blood disorder chronic ITP.

In February 2011, the FDA granted GSK full approval status for Promacta for ITP in the United States following the submission of long-term safety data from post-marketing clinical studies, as well as the completion of other commitments that verify the clinical benefit to patients. Additionally, it was reported in November 2011 that the Risk Evaluation and Mitigation Strategies (REMS) program that Promacta had been operating under in the United States was being significantly reduced in scope by the FDA due to data that had been submitted by GSK demonstrating the long-term safety of Promacta.

In November 2012, the FDA approved Promacta for the treatment of thrombocytopenia (low blood platelet counts) in patients with chronic hepatitis C to allow them to initiate and maintain interferon-based therapy. Promacta is the first supportive care treatment available to patients who are ineligible or poor candidates for interferon-based therapy due to their low blood platelet counts. Promacta in combination with interferon-based therapy has been shown to improve a patient's chance of achieving a sustained virologic response (SVR) or viral cure.

In September 2013, GSK received Marketing Authorization from the European Commission for an additional Revolade (eltrombopag/Promacta) indication as the first approved treatment for chronic Hepatitis C-associated thrombocytopenia.

GSK is conducting clinical studies of Promacta for various indications, including oncology-related indications. Promacta is authorized for use in 95 countries.

We entered into a Research, Development and License Agreement with SmithKline Beecham Corporation (now GSK) on December 29, 1994. The purpose of the agreement was to engage in a joint research and development effort to discover and/or design small molecule compounds which act as modulators of certain signal transducers and activators of transcription, or STATS, to develop pharmaceutical products from such compounds and to commercialize products resulting from the joint research and development. We granted an exclusive license under our patent rights to any product developed from the joint research. GSK has listed a patent in the FDA's Orange Book for Promacta with an expiration date in 2027, and we are entitled to receive royalties related to Promacta under this license as set forth below. The obligation to pay royalties lasts during the life of the relevant patents or at a reduced rate for ten years from the first commercial sale, whichever is longer, on a country-by-country basis. Absent early termination for bankruptcy or material breach, the term of the agreement expires upon expiration of the obligation to pay royalties. Either party may terminate the agreement in the event of bankruptcy or material breach. There are no remaining milestones to be paid under the agreement. We are entitled to receive royalties on annual net sales of Promacta as set forth in the following table:





Table of Contents

AGGREGATE NET SALES IN EACH CALENDAR YEAR	ROYALTY RATE*	
On portion of sales less than \$100 million	4.7	%
On portion of sales in range of \$100 million to \$200 million	6.6	%
On portion of sales in range of \$200 million to \$400 million	7.5	%
On portion of sales in range of \$400 million to \$1.5 billion	9.4	%
On portion of sales greater than \$1.5 billion	9.3	%

\*Net royalties due Ligand after payment to Rockefeller University.

Any such royalties may be subject to reduction (e.g., in the event of no patent coverage for the product) and/or may be subject to other terms and conditions set forth in our license agreement with GSK.

Kyprolis (Onyx Pharmaceuticals, a subsidiary of Amgen)

Ligand and Onyx Pharmaceuticals (formerly Proteolix, and now a subsidiary of Amgen, Inc.), entered into a collaboration in 2005 to develop the Captisol-enabled IV formulation of the active ingredient carfilzomib for refractory multiple myeloma. Under this agreement we agreed to sell Captisol to Onyx for use with carfilzomib, and granted an exclusive product-specific license under our patent rights with respect to Captisol. In July 2012, Onyx received accelerated approval from the FDA for Kyprolis (carfilzomib) for injection. Kyprolis is formulated with Ligand's Captisol technology and is used for the treatment of patients with multiple myeloma who have received at least two prior therapies, including bortezomib and an immunomodulatory agent, and have demonstrated disease progression on or within 60 days of completion of the last therapy. The indication for Kyprolis is based on response rate.

Onyx's obligation to pay royalties does not expire until four years after the expiration of the last-to-expire patent covering Captisol. Our patents and applications relating to the Captisol component of Kyprolis are not expected to expire until 2033. Our agreement with Onyx may be terminated by either party in the event of material breach or bankruptcy, or unilaterally by Onyx with prior written notice, subject to certain surviving obligations such as placing orders under any binding forecasts. Absent early termination, the agreement will terminate upon expiration of the obligation to pay royalties. Under this agreement, we are entitled to receive remaining milestones of up to \$2.5 million, revenue from clinical and commercial Captisol material sales and royalties on annual net sales of Kyprolis as set forth in the following table:

AGGREGATE NET SALES IN EACH CALENDAR YEAR	ROYALTY RATE	
Up to, and including \$250 million	1.5	%
Above \$250 million to \$500 million	2.0	%
Above \$500 million to \$750 million	2.5	%
Above \$750 million	3.0	%

Avinza (Pfizer)

We currently receive royalty revenues from Pfizer, Inc., or Pfizer, for sales of the pain therapeutic Avinza®. In February 2007, we completed the sale of our Avinza product line, together with all patent rights and licenses related to Avinza, to King Pharmaceuticals, which was acquired by Pfizer in February 2011. As a result of the sale, we are entitled to receive royalties from Pfizer on net sales of Avinza through the term of the relevant patent, which we currently expect to expire on November 25, 2017. Royalties on annual net sales are paid at a rate of 5% on sales up to \$200 million, 10% on sales above \$200 million and 15% on sales above \$250 million. Neither party to the agreement has any ongoing termination rights.

We have multiple partnered programs in our portfolio that are either in or nearing the regulatory approval process. These programs represent the next series of potential royalty generating assets in our portfolio:

Captisol-enabled Melphalan IV (Spectrum Pharmaceuticals, Pivotal, Stem Cell Transplant Conditioning)

In March 2013, we licensed the full world-wide rights to Captisol-enabled melphalan IV to Spectrum Pharmaceuticals, Inc., or Spectrum. The Captisol-enabled, PG-free melphalan program uses a new intravenous formulation of melphalan for the multiple myeloma transplant setting, and has been granted Orphan Designation by

the

4

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## Table of Contents

FDA. The formulation avoids the use of propylene glycol, which has been reported to cause renal and cardiac side-effects that limit the ability to deliver higher quantities of therapeutic compounds. The use of the Captisol technology to reformulate melphalan is anticipated to allow for longer administration durations and slower infusion rates, potentially enabling clinicians to safely achieve a higher dose intensity of pre-transplant chemotherapy. Under the terms of the license agreement, we granted an exclusive license to Spectrum under our patent rights to Captisol relating to the melphalan product. We are eligible to receive over \$50 million in potential milestone payments under this agreement, and we are also eligible to receive royalties on future net sales of the Captisol-enabled melphalan product at a royalty rate in the range of 15% to 25%. Spectrum's obligation to pay royalties will expire at the end of the life of the relevant patents or when a competing product is launched, whichever is earlier, but in no event within ten years of the commercial launch. Our patents and applications relating to the Captisol component of melphalan are not expected to expire until 2033. Absent early termination, the agreement will terminate upon expiration of the obligation to pay royalties. The agreement may be terminated by either party for an uncured material breach or unilaterally by Spectrum by prior written notice. This program has completed enrollment in a pivotal clinical trial.

### Biologic Therapeutics Platform (Various Stages of Development)

In April 2013, we acquired a portfolio of possible future royalty and milestone payment rights from Selexis SA, based on over 15 Selexis commercial license agreement programs with various pharmaceutical companies. Under the terms of our Royalty Stream and Milestone Payments Purchase Agreement with Selexis, we are eligible to receive approximately \$17 million in milestones and potentially over \$40 million in estimated annual royalties from these assets. The payment obligations for the particular programs are set forth in the various underlying commercial license agreements between Selexis and various third parties, which have remaining terms tied to the life of the underlying patents, which we currently expect to be maintained until at least 2026. In return for the rights to these payment streams, we paid Selexis \$3.5 million in an upfront cash payment, and expect to make a \$1 million cash payment in April 2014 on the first anniversary of the acquisition. Neither we nor Selexis has any ongoing termination rights with respect to our acquisition agreement.

The programs that we acquired in this transaction are based on Selexis' technology platform for cell line development and scale-up to manufacturing of therapeutic proteins, and relate to pre-commercialized drugs that are currently being developed; the programs should thus require no funding or technological support from Ligand. Selexis retained ownership of the underlying intellectual property for each of these programs. The programs covered by the Selexis transaction include novel biologics programs with Merrimack (MM-121, MM-111, MM-302 and MM-151), Baxter (BAX69), Aveo, CSL and Glenmark and biosimilar programs with Coherus and Biocad.

### Select Other Late-Stage Development or Commercial Programs

Duavee (bazedoxifene/conjugated estrogens) and Viviant/Conbriza (Pfizer)

In 2010, our partner Pfizer launched Viviant® (bazedoxifene) in Japan for the treatment of postmenopausal osteoporosis. The drug is also marketed in Spain under the brand name Conbriza® through a co-promotion with Almirall, an international pharmaceutical company based in Spain. Viviant was approved in 2009 by the European Commission (under the trade name Conbriza) for the treatment of postmenopausal osteoporosis in women at increased risk of fracture. Viviant, a selective estrogen receptor modulator, or SERM, is a result of the successful research collaboration between Wyeth (now a subsidiary of Pfizer) and us that began in 1994. Pfizer is responsible for the registration and worldwide marketing of bazedoxifene, a synthetic drug specifically designed to reduce the risk of osteoporotic fractures while also protecting uterine tissue.

Pfizer has combined bazedoxifene (discussed above) with the active ingredient in Premarin® to create Duavee®, a combination therapy for the treatment of post-menopausal symptoms in women. Pfizer obtained FDA approval for Duavee in the United States in October 2013 and filed an approval submission with the EMA in 2012. Pfizer launched Duavee in the United States in the first quarter of 2014.

Net royalties on annual net sales of Viviant and Duavee are each payable to us at a rate shown in the table below and are payable through the life of the relevant patents.



Table of Contents

AGGREGATE NET SALES IN EACH CALENDAR YEAR	ROYALTY RATE *	
On portion of sales less than \$400 million	0.5	%
On portion of sales in range of \$400 million to \$1 billion	1.5	%
On portion of sales greater than \$1 billion	2.5	%

\* Net royalties due Ligand after payment to Royalty Pharma.

Any such royalties may be subject to reduction or offset for past milestone payments and/or may be subject to other terms and conditions set forth in our license agreement with Pfizer.

**Nexterone (Baxter International)**

In 2006, we outlicensed Nexterone, an injectable formulation combining amiodarone and Captisol, to Baxter International, Inc. or Baxter (which acquired Prism Pharmaceuticals, Inc., the original licensee, in 2011). Under the terms of the agreement, Baxter is responsible, under an exclusive worldwide license, for all development and commercialization of Nexterone at its sole expense. In 2010, Nexterone was approved by the FDA and launched in the United States in 2011. We are supplying Captisol to Baxter for use in accordance with the terms of the license agreement under a separate supply agreement. Baxter has paid milestone payments and is obligated to pay royalties to us on sales of Nexterone through early 2033.

**Captisol-enabled Noxafil-IV (Merck, NDA)**

We and Merck entered into a Captisol supply agreement in June 2011 for Captisol-enabled Noxafil-IV. Merck has completed a Phase 3 study for this program and it filed a 505(b)(2) in 2013 for approval in the United States and European Union to market the drug. In the United States, the New Drug Application, or NDA, for Noxafil-IV was filed and received FDA Priority Review in November 2013. In the European Union, the Marketing Authorization Application, or MAA, is filed with the European Medicines Agency. Action is expected for both the NDA and MAA in 2014, which may lead to commercial sale of Captisol for the program in multiple markets. We will receive our commercial compensation for this program through the sale of Captisol, and we will not receive a royalty on this program.

**MK-8931 Beta-Secretase Inhibitor (Merck, Phase 3, Alzheimer's Disease)**

We have a development agreement with Merck (formerly Schering-Plough) for a beta-secretase, or BACE, inhibitor program for the treatment of Alzheimer's disease. This disease is characterized by plaques of the toxic amyloid-beta protein within the brain. BACE is believed to be a key enzyme in the production of amyloid-beta protein.

Amyloid-beta is formed when the larger amyloid precursor protein (APP) is cleaved by two enzymes, BACE and gamma-secretase, which releases the amyloid-beta fragment. A BACE inhibitor is expected to reduce amyloid-beta generation in Alzheimer's disease patients.

In December 2012, Merck initiated a Phase 2/3 clinical trial for its lead BACE inhibitor product candidate, MK-8931, evaluating its safety and efficacy in patients with mild-to-moderate Alzheimer's disease. In December 2013, Merck announced progression of the program to Phase 3 by advancing the Phase 2/3 trial to Phase 3 and by initiating a second Phase 3 trial. We are entitled to a royalty on potential future sales by Merck.

**Sparsentan (formerly RE-021) (Retrophin, Phase 2, FSGS)**

In early 2012, we licensed the world-wide rights to Sparsentan (formerly known as RE-021 and DARA-a Dual Acting Receptor Antagonist of Angiotension and Endothelin receptors) to Retrophin, Inc., or Retrophin. Retrophin is developing Sparsentan for orphan indications of severe kidney diseases including FSGS as well as conduct proof-of-concept studies in resistant hypertension and diabetic nephropathy. Certain patient groups with severely compromised renal function exhibit extreme proteinuria resulting in progression to dialysis and a high mortality rate. Sparsentan, with its unique dual blockade of angiotensin and endothelin receptors, is expected to provide meaningful clinical benefits in mitigating proteinuria in indications where there are no approved therapies. Retrophin announced initiation of a potentially pivotal Phase 2 clinical trial for Sparsentan on January 2, 2014.

In late 2012, we received a milestone payment of 620,000 shares of common stock in Retrophin. Former license holders are entitled to receive 15% of the proceeds received upon sale of this stock, and all proceeds related to this



Table of Contents

program. Under our license agreement with Retrophin we are entitled to receive over \$75 million in milestones, as well as 9% in royalties on future worldwide sales by Retrophin through the life of the relevant patents, which we currently expect to be through at least 2019 and may be extended until 2024. In 2013 we received a net \$1.2 million milestone payment from Retrophin.

**Lasofoxifene (Azure Biotech and Ethicor, Estrogen receptor modulator)**

On July 17, 2013, we entered into a license agreement with Azure Biotech, Inc., or Azure. Under the agreement, we granted to Azure an exclusive worldwide license to develop and market a novel formulation of lasofoxifene. We are entitled to receive up to \$2.6 million in potential development and regulatory milestones as well as a 5% royalty on future net sales through the later of the life of the relevant patents (currently expected to be at least until 2027) or 10 years after regulatory approval. Azure may terminate the license agreement at any time upon six months' prior notice. Lasofoxifene is an estrogen partial agonist for osteoporosis treatment and other diseases, discovered through the research collaboration between us and Pfizer. Under the terms of the license agreement with Azure, we retain the rights to the oral formulation of lasofoxifene originally developed by Pfizer.

In July 2013, we also entered into a license agreement with Ethicor for the manufacture and distribution of the oral formulation of lasofoxifene in the European Economic Area, Switzerland and the Indian Subcontinent. Under the terms of the agreement, we are entitled to receive potential sales milestones of up to \$16 million and a 25% royalty on future net sales. Ethicor plans to supply oral lasofoxifene as an unlicensed medicinal product, which may be requested by healthcare professionals to meet the clinical needs of patients when authorized medicines are unsuitable or contraindicated. In the European Union, there are approximately 37 million women with osteoporosis.

**Captisol-enabled Carbamazepine-IV (Lundbeck, Phase 3, Epilepsy)**

We have a development and commercialization agreement for Captisol-enabled carbamazepine-IV with Lundbeck (formerly Ovation Pharmaceuticals) for the use of Captisol in the formulation of CE carbamazepine-IV. Lundbeck is developing CE carbamazepine-IV for the management of acute seizure disorder for hospital or emergency settings and announced plans to submit an NDA prior to the end of 2013.

**Captisol-enabled Delafloxacin-IV (Melinta, Phase 3, Infection)**

We entered into a development and commercialization agreement for Captisol-enabled delafloxacin-IV in 2008 with Melinta Therapeutics, Inc. (formerly Rib-X Pharmaceuticals), or Melinta, for the use of Captisol in the intravenous formulation of delafloxacin. Delafloxacin is a novel hospital-focused fluoroquinolone antibiotic candidate with potency against a variety of quinolone-resistant Gram-positive and Gram-negative bacteria, including quinolone-resistant, methicillin-resistant *Staphylococcus aureus*, or MRSA. In 2013 Melinta initiated the first of two planned Phase 3 clinical trials of delafloxacin for the treatment of acute bacterial skin and skin structure infections (ABSSSI), including infections caused by MRSA. Melinta has made certain milestone payments to us already and may be required to pay us an aggregate of an additional \$3.6 million upon the achievement of specified development and regulatory approval milestones. We are entitled to a royalty on potential future sales by Melinta.



Table of Contents

## Captisol-enabled Topiramate IV (CurX, Phase 1, Epilepsy)

In July 2013, the FDA granted orphan-drug designation for our proprietary Captisol-enabled Topiramate Injection for the treatment of partial onset or primary generalized tonic-clonic seizures in hospitalized epilepsy patients who are unable to take oral topiramate. In August 2013, we entered a global license agreement with CURx Pharmaceuticals, Inc. for the development and commercialization of Topiramate. CurX has made certain milestone payments to us already and may be required to pay us an aggregate of an additional \$19.6 million, net of amounts owed to third parties upon the achievement of specified milestones. Additionally, we are owed net tiered royalties on future sales of 6.0% to 7.5%.

## Internal Product Development Programs

As summarized in the table below, we are developing several proprietary products for a variety of indications. These programs represent our future licensing opportunities to expand our partnered asset portfolio.

Program	Disease/Indication	Development Phase
HepDirect	Liver Diseases	Preclinical
Oral Human Granulocyte Colony Stimulating Factor	Neutropenia	Preclinical
IRAK-4	Inflammation	Preclinical
Glucagon Receptor Antagonist	Diabetes	Phase 1
Selective Androgen Receptor Modulator	Various	Phase 2-ready
Captisol-Enabled Clopidogrel	Anti-coagulant	Phase 3

## HepDirect HCV Inhibitor Program

We are developing novel small molecule inhibitors of the Hepatitis C virus using our HepDirect technology platform. Data from current lead molecules suggest that directing these molecules to the liver using the HepDirect technology could produce fewer side effects and has the potential for an overall superior risk-benefit ratio compared to non-HepDirect therapies.

## Oral Human Granulocyte Colony Stimulating Factor (GCSF) Program

We have discovered a novel series of small molecules that selectively activate human granulocyte colony stimulating factor, or GCSF, receptor function in a manner distinct from GCSF, but similar to the mechanism of small-molecule human thrombopoietin receptor (hTPOR) agonists, such as eltrombopag (Promacta). The goal of our GCSFR agonist program is to develop a non-peptide, small molecule, oral GCSFR agonist that is a convenient, cost-effective alternative as compared to recombinant human GCSF for the treatment of neutropenia and other related indications. The lead compound, LG7455, activates the GCSF-GCSFR signaling pathway and induces the differentiation of human bone marrow cells into granulocytes. It also significantly increases peripheral blood neutrophils and demonstrated the first reported proof-of-concept for a small molecule GCSF receptor antagonist in a primate model. Further optimization of the LG7455 structure series could lead to a first-in-class, once-daily, oral medication for the treatment of congenital, chronic or chemotherapy-induced neutropenia.

## IRAK4 Inhibitor Program

We are developing small molecule Interleukin-1 Receptor Associated Kinase-4, or IRAK4, inhibitors for the treatment of inflammatory and immune disorders. IRAK4 plays an important role in the innate immune system and may also be

important for cross-talk between the innate and adaptive immune systems. IRAK4 is a key signaling component downstream of both toll-like receptors and interleukin-1 receptors suggesting that it may have therapeutic value for a range of autoimmune and inflammatory conditions. Inhibition of IRAK4 activity has been implicated in multiple diseases including rheumatoid arthritis, systemic lupus erythematosus, gout, inflammatory bowel disease, asthma, and allergic rhinitis. Inhibitors of IRAK4 may also be useful for the treatment of certain leukemias and lymphomas. We have identified orally available small molecule inhibitors of IRAK4 which are under investigation for use in cancer and autoimmune diseases.

## Table of Contents

### Glucagon Receptor Antagonist Program

We are currently developing small molecule glucagon receptor antagonists for the treatment of Type II diabetes mellitus. Compounds that block the action of glucagon may reduce the hyperglycemia that is characteristic of this disease. Glucagon stimulates the production of glucose by the liver and its release into the blood stream. In diabetic patients, glucagon secretion is abnormally elevated and contributes to hyperglycemia in these patients. Clinical proof of concept studies with glucagon receptor antagonists in Type 2 diabetic patients were reported at the American Diabetes Association Annual Meeting in 2011 and 2012, supporting the potential benefit of this therapeutic target. Our advanced glucagon antagonist compound blocks glucagon action in human hepatocytes in vitro, reduces blood glucose in animal models of Type 1 and Type 2 diabetes, has demonstrated good oral bioavailability in rodents, and has a safety profile in preclinical studies suitable for further clinical development.

In October 2013, the FDA accepted our Investigational New Drug, or IND, application for our proprietary Glucagon receptor antagonist product (LGD-6972) candidate for the treatment of diabetes. LGD-6972 was acquired in connection with our acquisition of Metabasis and we may be required to remit payment to the contingent value right, or CVR, holders upon the sale or partnering of the asset. We initiated a Phase 1 clinical trial in the fourth quarter of 2013.

### Selective Androgen Receptor Modulator (SARM)

Our LGD-4033 is a non-steroidal selective androgen receptor modulator, or SARM, that is expected to produce the therapeutic benefits of testosterone with improved safety, tolerability and patient acceptance due to a tissue-selective mechanism of action and an oral route of administration. We have discovered several novel orally active, non-steroidal SARM compounds, including LGD-4033, based on tissue-specific gene expression and other functional, cell-based technologies. In animal models, LGD-4033 demonstrated anabolic activity in muscles, anti-resorptive and anabolic activity in bones and a robust selectivity for muscle and bone versus prostate and sebaceous glands. Phase 1 single and multiple dose escalation studies of LGD-4033 were conducted in a total of 116 healthy male subjects. The safety, tolerability and preliminary efficacy of LGD-4033 was evaluated in the double-blind, placebo-controlled Phase 1 multiple ending dose study. Healthy male subjects were randomized to receive 0.1, 0.3 or 1.0 mg LGD-4033 or placebo once daily over 21 days. Key findings of this study included: LGD-4033 was safe and well tolerated at all doses following daily oral administration for three weeks in young healthy males; no clinically significant dose-related adverse events were reported; no clinically significant changes in liver function tests, PSA, hematocrit or ECG were seen; positive dose-dependent trends in lean muscle mass increase were observed with drug-treated subjects; positive dose-dependent trends in functional exercise and strength measures were consistent with anabolic activity. LGD-4033 is positioned to enter into Phase 2 development, and potential studies include evaluation of LGD-4033 in conditions such as muscle wasting associated with cancer (cachexia), acute rehabilitation (e.g. hip fracture), and acute illness.

### Captisol-Enabled Clopidogrel (Unpartnered, Phase 3, Anti-coagulant)

Clopidogrel is the active ingredient in PLAVIX®, a leading anti-platelet medication which is currently only available in an oral formulation. The Captisol-enabled Clopidogrel formulation is designed to provide an intravenous option in situations where the administration of oral platelet inhibitors is not feasible or desirable. We licensed the full worldwide rights to The Medicines Company, or MedCo, in June 2011. In July 2013, we and MedCo mutually terminated the License Agreement dated June 1, 2011 and the related Supply Agreement dated June 1, 2011. Upon termination, the licensed rights relating to the compound were returned to us. MedCo recently conducted a pharmacokinetic and pharmacodynamic study of oral Clopidogrel and Captisol-enabled intravenous Clopidogrel in healthy volunteers. The study indicated a potential difference in metabolism between the oral and intravenous routes of administration for Clopidogrel, and MedCo elected not to proceed with further development.



## Table of Contents

Other Internal Programs Eligible for Further Development Funding, Either Through Ligand or a Partner

▲Aplindore (Phase 2, Restless Leg/Parkinson's)  
●Captisol-enabled Nasal Budesonide (Phase 1, Allergic Rhinitis)  
■Thyroid Receptor-beta Agonist (Phase 1, Dyslipidemia)  
■Histamine H3 Receptor Antagonist (Preclinical, Cognitive Disorders)  
●Glucokinase Activator (Preclinical, Diabetes)  
■DGAT Inhibitor (Preclinical, Diabetes)  
●CCR1 Inhibitor (Preclinical, Oncology)  
●CRTH2 Inhibitor (Preclinical, Inflammation)  
■Topical JAK3 (Preclinical, Inflammation)  
●Oral Erythropoietin (Preclinical, Anemia)  
■Meloxicam (Preclinical, Pain)

●Others

### Technology

We employ various research laboratory methods to discover and conduct preclinical development of new chemical entities. These methods are performed either in our own laboratories or in those of contract research organizations under our direction.

Our discovery work is based on certain technologies and acquired special expertise related to intracellular receptors and the receptors for hematopoietic growth factors. Intracellular receptors are involved in the actions of non-peptide hormones and drugs such as SERMs and SARMs. Hematopoietic growth factor receptors are involved in the differentiation and proliferation of blood cell progenitors, the formation of new blood cells, and the action of drugs such as Promacta, Epogen and Neumega. We use and have developed particular expertise in co-transfection assays, which measure gene transcription in response to the activation of a target receptor, and gene expression in cells selected for expression of particular receptors or transfected with cDNA for particular receptors. Some of these methods are covered by patents issued to or licensed by us, some are trade secrets, and some are methods that are in the public domain, but that we may use in novel ways to improve our efficiency in identifying promising leads and developing new chemical entities.

In connection with our merger with Metabasis, we acquired certain HepDirect technology. HepDirect technology supplements our core drug discovery technology platform of ligand-dependent gene expression. HepDirect is a prodrug technology that targets delivery of certain drugs to the liver by using a proprietary chemical modification that renders a drug biologically inactive until cleaved by a liver-specific enzyme.

In connection with our acquisition of CyDex, we acquired the Captisol drug formulation platform technology. We use this technology to improve the solubility, stability, and/or pharmacokinetics of drugs, whether in our own internal development pipeline or those of our partners.

### Manufacturing

We currently have no manufacturing facilities and rely on third parties, including our collaborative partners, for clinical production of any products or compounds.

We currently outsource the production of Captisol to Hovione FarmaCiencia SA, or Hovione, a major supplier of active pharmaceutical ingredients, or APIs and API intermediates located in Portugal. In 2002, CyDex entered into a Captisol supply agreement with Hovione, under which Hovione is our exclusive supplier of Captisol and is restricted from supplying Captisol to third parties, so long as specified conditions are met. In addition to its main manufacturing site in Loures, Portugal, Hovione will qualify additional sites if our forecast requirements for Captisol exceed the capabilities of the Loures site. We have ongoing minimum purchase commitments under the agreement and are required

## Table of Contents

to pay Hovione an aggregate minimum amount during the agreement term. In 2008, we entered into an amendment to the supply agreement, under which we and Hovione agreed to reduce our minimum annual purchase requirement of Captisol and to extend the term of the agreement.

We pay Hovione unit prices, in U.S. dollars, for all Captisol supplied, which prices may be adjusted for fluctuation in currency exchange rates, change in raw material prices and change in the Portuguese consumer price index.

Additionally, prices may be adjusted based on requested changes to the Captisol manufacturing process or specifications.

In the event of a Captisol supply interruption, we are permitted to designate and, with Hovione's assistance, qualify one or more alternate suppliers. If the supply interruption continues beyond a designated period, we may terminate the agreement. In addition, if Hovione cannot supply our requirements of Captisol due to an uncured force majeure event or if the unit price of Captisol exceeds a set figure, we may obtain Captisol from a third party. In December 2011, the contract was amended to allow certain bulk quantities of Captisol to be distributed directly from Hovione.

Additionally, in 2012, we qualified a Hovione site in Cork, Ireland to perform certain manufacturing steps to provide back-up and increased capacity to the Loures site.

The initial term of the agreement expires in December 2019. The agreement will automatically renew for successive two year renewal terms unless either party gives written notice of its intention to terminate the agreement no less than two years prior to the expiration of the initial term or renewal term. In addition, either party may terminate the agreement for the uncured material breach or bankruptcy of the other party or an extended force majeure event. We may terminate the agreement for extended supply interruption, regulatory action related to Captisol or other specified events.

For further discussion of these items, see below under "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations."

### Competition

Some of the drugs we and our collaborative partners are developing may compete with existing therapies or other drugs in development by other companies. A number of pharmaceutical and biotechnology companies are pursuing intracellular receptor-related approaches to drug discovery and development. Furthermore, academic institutions, government agencies and other public and private organizations conducting research may seek patent protection with respect to potentially competing products or technologies and may establish collaborative arrangements with our competitors.

Many of our existing or potential competitors, particularly large pharmaceutical companies, have greater financial, technical and human resources than we do and may be better equipped to develop, manufacture and market products. Many of these companies also have extensive experience in preclinical testing and human clinical trials, obtaining FDA and other regulatory approvals and manufacturing and marketing pharmaceutical products.

Our competitive position also depends upon our ability to attract and retain qualified personnel, obtain patent protection or otherwise develop proprietary products or processes, and secure sufficient capital resources for the often substantial period between technological conception and commercial sales. For a discussion of the risks associated with competition, see below under "Item 1A. Risk Factors."

### Government Regulation

The manufacturing and marketing of our products, our ongoing research and development activities and products being developed by our collaborative partners are subject to regulation for safety and efficacy by numerous governmental authorities in the United States and other countries. In the United States, pharmaceuticals are subject to rigorous regulation by federal and various state authorities, including the FDA. The Federal Food, Drug and Cosmetic Act and the Public Health Service Act govern the testing, manufacture, safety, efficacy, labeling, storage, record keeping, approval, advertising and promotion of our products. There are often comparable regulations that apply at the state level. Product development and approval within this regulatory framework takes a number of years and involves the expenditure of substantial resources.

## Table of Contents

The steps required before a pharmaceutical agent may be marketed in the United States include (1) preclinical laboratory tests, (2) the submission to the FDA of an IND, which must become effective before human clinical trials may commence, (3) adequate and well-controlled human clinical trials to establish the safety and efficacy of the drug, (4) the submission of an NDA to the FDA and (5) the FDA approval of the NDA prior to any commercial sale or shipment of the drug. In addition to obtaining FDA approval for each product, each domestic drug-manufacturing establishment must be registered with the FDA and, in California, with the Food and Drug Branch of California. Domestic manufacturing establishments are subject to pre-approval inspections by the FDA prior to marketing approval, then to biennial inspections, and must comply with current Good Manufacturing Practices (cGMP). To supply products for use in the United States, foreign manufacturing establishments must comply with cGMP and are subject to periodic inspection by the FDA or by regulatory authorities in such countries under reciprocal agreements with the FDA.

For both currently marketed and future products, failure to comply with applicable regulatory requirements after obtaining regulatory approval can, among other things, result in the suspension of regulatory approval, as well as possible civil and criminal sanctions. In addition, changes in existing regulations could have a material adverse effect on us.

We are also increasingly subject to regulation by the states. A number of states now regulate, for example, pharmaceutical marketing practices and the reporting of marketing activities, controlled substances, clinical trials and general commercial practices. We have developed and are developing a number of policies and procedures to ensure our compliance with these state laws, in addition to the federal regulations described above. Significant resources are now required on an ongoing basis to ensure such compliance. For a discussion of the risks associated with government regulations, see below under “Item 1A. Risk Factors.”

### Patents and Proprietary Rights

We believe that patents and other proprietary rights are important to our business. Our policy is to file patent applications to protect technology, inventions and improvements to our inventions that are considered important to the development of our business. We also rely upon trade secrets, know-how, continuing technological innovations and licensing opportunities to develop and maintain our competitive position.

Patents are issued or pending for the following key products or product families. The scope and type of patent protection provided by each patent family is defined by the claims in the various patents. The nominal patent expiration dates have been provided. The actual patent term may vary by jurisdiction and depend on a number of factors including potential patent term adjustments, patent term extensions, and terminal disclaimers. For each product or product family, the patents and/or applications referred to are in force in at least the United States, and for most products and product families, the patents and/or applications are also in force in European jurisdictions, Japan and other jurisdictions.

#### Promacta

Patents covering Promacta are owned by GSK. The United States patent listed in the FDA’s listing of Approved Drug Products with Therapeutic Equivalence Evaluations (the “Orange Book”) relating to Promacta with the latest expiration date is not expected to expire until 2027. The type of patent protection (e.g., composition of matter or use) for each patent listed in the Orange Book and the expiration date for each patent listed in the Orange Book are provided in the following table. In addition, certain related patents in the commercially important jurisdictions of Europe and Japan are identified in the following table.

Table of Contents

U.S. Patent No.	U.S. Expiration Date	Type of Protection	Jurisdiction (Expiration Date)§
U.S. 6,280,959	Oct. 30, 2018	composition of matter and use	EP 1864981 (05/24/21)
U.S. 7,160,870	Nov. 20, 2022	composition of matter and use	EP 1294378 (05/24/21) JP 3813875 (05/24/21)
U.S. 7,332,481	May 24, 2021	use	EP 1889838 (05/24/21) JP 4546919 (05/24/21)
U.S. 7,452,874	May 24, 2021	composition of matter and use	EP 1889838 (05/24/21) JP 4546919 (05/24/21)
U.S. 7,473,686	May 24, 2021	composition of matter and use	EP 1864981 (05/24/21) EP 1294378 (05/24/21) JP 3813875 (05/24/21)
U.S. 7,547,719	Jul. 13, 2025	composition of matter and use	EP 1534390 (05/21/23) JP 4612414 (05/21/23)
U.S. 7,790,704	May 24, 2021	use	
U.S. 7,795,293	May 21, 2023	use	
U.S. 8,052,993	Aug. 1, 2027	composition of matter and use	
U.S. 8,052,994	Aug. 1, 2027	composition of matter and use	
U.S. 8,052,995	Aug. 1, 2027	composition of matter and use	
U.S. 8,062,665	Aug. 1, 2027	composition of matter and use	
U.S. 8,071,129	Aug. 1, 2027	composition of matter and use	

Expiration dates of European and Japanese patents are calculated as 20 years from the earliest nonprovisional filing date to which priority is claimed, and do not take into account extensions that are or may be available in these jurisdictions.

## Kyprolis

Patents protecting Kyprolis include those owned by Onyx Pharmaceuticals and those owned by Ligand. The United States patent listed in the Orange Book relating to Kyprolis with the latest expiration date is not expected to expire until 2027. Patents and applications owned by Ligand relating to the Captisol component of Kyprolis are not expected to expire until 2033. The type of patent protection (e.g., composition of matter or use) for each patent listed in the Orange Book and the expiration dates for each patent listed in the Orange Book are provided in the following table. In addition, certain related patents in the commercially important jurisdictions of Europe and Japan are identified in the following table.

U.S. Patent No.	U.S. Expiration Date	Type of Protection	Jurisdiction (Expiration Date)§
U.S. 7,232,818	Apr. 14, 2025	composition of matter	EP 1745064 (04/14/25)
U.S. 7,417,042	Jun. 7, 2026	composition of matter	EP 1781688 (08/08/25) JP 4743720 (08/08/25)
U.S. 7,491,704	Apr. 14, 2025	use	EP 1745064 (04/14/25) EP 1819353 (12/07/25) EP 2260835 (12/07/25)
U.S. 7,737,112	Dec. 7, 2027	composition of matter	JP 4990155 (12/07/25) JP 5108509 (05/09/25)
U.S. 8,129,346	Dec. 25, 2026	use	EP 1745064 (04/14/25)
U.S. 8,207,125	Apr. 14, 2025	composition of matter	EP 1781688 (08/08/25) JP 4743720 (08/08/25)
U.S. 8,207,126	Apr. 14, 2025	composition of matter and use	
U.S. 8,207,127	Apr. 14, 2025	use	
U.S. 8,207,297	Apr. 14, 2025	composition of matter and use	



Expiration dates of European and Japanese patents are calculated as 20 years from the earliest nonprovisional filing date to which priority is claimed, and do not take into account extensions that are or may be available in these jurisdictions.

Table of Contents

## Avinza

The United States patent listed in the Orange Book relating to the Avinza formulation with the latest expiration date is not expected to expire until 2017; however, applications for generic forms of Avinza have been submitted to the FDA. The type of patent protection (e.g., composition of matter or use) for the patent listed in the Orange Book and the expiration date for the patent is provided in the following table. Certain related patents in other jurisdictions are not identified in the following table, as our royalties are based on sales in the United States.

U.S. Patent No.	U.S. Expiration Date	Type of Protection
U.S. 6,066,339	Nov. 25, 2017	composition of matter

## Captisol

Patents and pending patent applications covering Captisol are owned by Ligand. Other patents and pending patent applications covering methods of making Captisol are owned by Ligand or by Pfizer. The patents covering the Captisol product, if issued, with the latest expiration date would not be set to expire until 2033 (see, e.g., WO 2013/130666 (contains composition of matter and use claims; filed Feb. 27, 2013)). Ligand also owns several patents and pending patent applications covering drug products containing Captisol as a component. The type of patent protection (e.g., composition of matter or use) and the expiration dates for several issued patents covering Captisol are provided in the following table. In addition, certain related patents and applications in the commercially important jurisdictions of Europe and Japan are listed in the following table.

U.S. Patent No.	U.S. Expiration Date	Type of Protection	Jurisdiction (Expiration Date) <sup>‡</sup>
U.S. 8,114,438	Mar. 19, 2028	composition of matter	EP 1755551 (pending) JP 2013028645 (pending)
U.S. 7,629,331	Oct. 26, 2025	composition of matter	EP 1945228 (10/26/25) EP 2581078 (pending)
U.S. 8,049,003	Dec. 19, 2026	use	EP 2583668 (pending) EP 2335707 (pending) EP 2268269 (pending)
U.S. 7,635,773	Mar. 13, 2029	composition of matter and use	JP 4923144 (04/28/29) JP 2012072160 (pending) EP 2268269 (pending)
U.S. 8,410,077	Sep. 6, 2030*	composition of matter	JP 4923144 (04/28/29) JP 2012072160 (pending)

<sup>‡</sup>Expiration date of European and Japanese patents are calculated as 20 years from the earliest nonprovisional filing date to which priority is claimed, and do not take into account extensions that are or may be available in these jurisdictions.

\*Expiration date is subject to a terminal disclaimer.

Subject to compliance with the terms of the respective agreements, our rights to receive royalty payments under our licenses with our exclusive licensors typically extend for the life of the patents covering such developments. For a discussion of the risks associated with patent and proprietary rights, see below under “Item 1A. Risk Factors.”

## Human Resources

As of February 1, 2014, we had 20 full-time employees, of whom 6 are involved directly in scientific research and development activities. Of these employees, 6 hold Ph.D. or M.D. degrees.

Table of Contents

ITEM 1A. RISK FACTORS

The following is a summary description of some of the many risks we face in our business. You should carefully review these risks in evaluating our business, including the businesses of our subsidiaries. You should also consider the other information described in this report.

Revenues based on Promacta and Kyprolis represent a substantial portion of our overall current and/or expected future revenues.

GSK is obligated to pay us royalties on its sales of Promacta and we receive revenue from Onyx based on both sales of Kyprolis and purchases of Captisol material for clinical and commercial uses. These payments are expected to be a substantial portion of our ongoing revenues for some time. As a result, any setback that may occur with respect to Promacta or Kyprolis could significantly impair our operating results and/or reduce the market price of our stock. Setbacks for Promacta and Kyprolis could include problems with shipping, distribution, manufacturing, product safety, marketing, government regulation or reimbursement, licenses and approvals, intellectual property rights, competition with existing or new products and physician or patient acceptance of the product, as well as higher than expected total rebates, returns or discounts.

Revenue from sales of Captisol material to our collaborative partners represents a significant portion of our current revenue and our continued development and supply of Captisol is subject to a number of risks.

In January 2011, we completed our merger with CyDex. All of CyDex's products and product candidates, as well as the technology that it outlicenses, are based on Captisol. As a result, any setback that may occur with respect to Captisol could significantly impair our operating results and/or reduce the market price of our stock. Setbacks for Captisol could include problems with shipping, distribution, manufacturing, product safety, marketing, government regulation or reimbursement, licenses and approvals, intellectual property rights, competition with existing or new products and physician or patient acceptance of the products using Captisol, as well as higher than expected total rebates, returns or discounts for such products.

If products or product candidates incorporating Captisol technology were to cause any unexpected adverse events, the perception of Captisol safety could be seriously harmed. If this were to occur, we may not be able to market Captisol products unless and until we are able to demonstrate that the adverse event was unrelated to Captisol, which we may not be able to do. Further, whether or not the adverse event was a result of Captisol, we could be required by the FDA to submit to additional regulatory reviews or approvals, including extensive safety testing or clinical testing of products using Captisol, which would be expensive and, even if we were to demonstrate that the adverse event was unrelated to Captisol, would delay our marketing of Captisol-enabled products and receipt of revenue related to those products, which could significantly impair our operating results and/or reduce the market price of our stock.

We obtain Captisol from a sole source supplier, and if this supplier were to cease to be able to supply Captisol to us, or decline to supply Captisol to us, we would be unable to continue to derive revenue or continue to develop our product candidates until we obtained an alternative source, which could take a considerable length of time. Our supplier of Captisol is Hovione, through its agent Hovione, LLC. If a major disaster were to happen at Hovione's facilities or Hovione were to suffer major production problems or were to fail to deliver Captisol to us for any other reason, there could be a significant interruption of our Captisol supply. A series of unusually large orders could rapidly deplete our inventory and cause significant problems with our licensees and disrupt our business. In addition, if we fail to meet certain of our obligations under our supply agreements, our customers could obtain the right to have Captisol manufactured by other suppliers, which would significantly harm our business.

We currently depend on our arrangements with our outlicensees to sell products using our Captisol technology. These agreements generally provide that outlicensees may terminate the agreements at will. If our outlicensees discontinue

sales of products using our Captisol technology, fail to obtain regulatory approval for products using our Captisol technology, fail to satisfy their obligations under their agreements with us, or choose to utilize a generic form of Captisol should it become available, or if we are unable to establish new licensing and marketing relationships, our financial results and growth prospects would be materially affected. We maintain inventory of Captisol, which has a five year shelf life, at three geographically spread storage locations in the US and Europe. If disasters were to strike one or all three of these locations, it could lead to supply interruptions. Further, under most of our Captisol outlicenses, the amount of royalties we receive will be reduced or will cease when the relevant patent expires. Our high purity patents, U.S. Patent Nos. 7,635,773 and 8,410,077 and foreign equivalents, are not expected to expire until 2029 and our morphology patents, U.S. Patent Nos. 7,629,331 and 8,049,003 and foreign equivalents, are not expected to expire until 2025, but the initially filed patents relating to Captisol expired starting in 2010 in the United States and will expire by 2016 in most countries outside the United States. If our other intellectual property rights

## Table of Contents

are not sufficient to prevent a generic form of Captisol from coming to market and if in such case our outlicensees choose to terminate their agreements with us, our Captisol revenue may decrease significantly

The product candidates of our partners and us face significant development and regulatory hurdles prior to partnering and/or marketing which could delay or prevent licensing, sales and/or milestone revenue.

Before we or our partners obtain the approvals necessary to sell any of our unpartnered assets or partnered programs, we must show through preclinical studies and human testing that each potential product is safe and effective. We and/or our partners have a number of partnered programs and unpartnered assets moving toward or currently awaiting regulatory action. Failure to show any product's safety and effectiveness could delay or prevent regulatory approval of a product and could adversely affect our business. The drug development and clinical trials process is complex and uncertain. For example, the results of preclinical studies and initial clinical trials may not necessarily predict the results from later large-scale clinical trials. In addition, clinical trials may not demonstrate a product's safety and effectiveness to the satisfaction of the regulatory authorities. Recently, a number of companies have suffered significant setbacks in advanced clinical trials or in seeking regulatory approvals, despite promising results in earlier trials. The FDA may also require additional clinical trials after regulatory approvals are received. Such additional trials may be expensive and time-consuming, and failure to successfully conduct those trials could jeopardize continued commercialization of a product.

The rates at which we complete our scientific studies and clinical trials depends on many factors, including, but are not limited to, our ability to obtain adequate supplies of the products to be tested and patient enrollment. Patient enrollment is a function of many factors, including the size of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial and other potential drug candidates being studied. Delays in patient enrollment for our trials may result in increased costs and longer development times. In addition, our collaborative partners have rights to control product development and clinical programs for products developed under our collaborations. As a result, these collaborative partners may conduct these programs more slowly or in a different manner than expected. Moreover, even if clinical trials are completed, we or our collaborative partners still may not apply for FDA approval in a timely manner or the FDA still may not grant approval.

We rely heavily on collaborative relationships, and any disputes or litigation with our collaborative partners or termination or breach of any of the related agreements could reduce the financial resources available to us, including milestone payments and future royalty revenues.

Our strategy for developing and commercializing many of our potential products, including products aimed at larger markets, includes entering into collaboration agreements with corporate partners and others. These agreements give our collaborative partners significant discretion when deciding whether or not to pursue any development program. Our existing collaborations may not continue or be successful, and we may be unable to enter into future collaborative arrangements to develop and commercialize our unpartnered assets.

In addition, our collaborators may develop drugs, either alone or with others that compete with the types of drugs they are developing with us (or that we are developing on our own). This would result in increased competition for our or our partners' programs. If products are approved for marketing under our collaborative programs, revenues we receive will depend on the manufacturing, marketing and sales efforts of our collaborative partners, who generally retain commercialization rights under the collaborative agreements. Generally, our current collaborative partners also have the right to terminate their collaborations at will or under specified circumstances. If any of our collaborative partners breach or terminate their agreements with us or otherwise fail to conduct their collaborative activities successfully (for example, by not making required payments when due, or at all), our product development under these agreements will be delayed or terminated. Disputes or litigation may also arise with our collaborators (with us and/or with one or more third parties), including disputes or litigation over ownership rights to intellectual property, know-how or technologies

developed with our collaborators. Such disputes or litigation could adversely affect our rights to one or more of our product candidates. Any such dispute or litigation could delay, interrupt or terminate the collaborative research, development and commercialization of certain potential products, create uncertainty as to ownership rights of intellectual property, or could result in litigation or arbitration. The occurrence of any of these problems could be time-consuming and expensive and could adversely affect our business.

Expirations of, challenges to or failure to secure patents and other proprietary rights may significantly hurt our business.

Any conflicts resulting from the patent rights of others could significantly reduce the coverage of our patents and limit our ability to obtain meaningful patent protection. We have had and will continue to have discussions with our current and potential collaborative partners regarding the scope and validity of our patents and other proprietary rights. If a collaborative partner or other party successfully establishes that our patent rights are invalid, we may not be able to continue our existing

## Table of Contents

collaborations beyond their expiration. Any determination that our patent rights are invalid also could encourage our collaborative partners to seek early termination of our agreements. Such invalidation could adversely affect our ability to enter into new collaborations.

We may also need to initiate litigation, which could be time-consuming and expensive, to enforce our proprietary rights or to determine the scope and validity of others' rights. If litigation occurs, a court may find our patents or those of our licensors invalid or may find that we have infringed on a competitor's rights. In addition, if any of our competitors have filed patent applications in the United States which claim technology we also have invented, the United States Patent and Trademark Office may require us to participate in expensive interference proceedings to determine who has the right to a patent for the technology.

We also rely on unpatented trade secrets and know-how to protect and maintain our competitive position. We require our employees, consultants, collaborative partners and others to sign confidentiality agreements when they begin their relationship with us. These agreements may be breached, and we may not have adequate remedies for any breach. In addition, our competitors may independently discover our trade secrets.

Generally, our success will depend on our ability and the ability of us and our licensors to obtain and maintain patents and proprietary rights for our potential products both in the United States and in foreign countries. Patents may not be issued from any of these applications currently on file, or, if issued, may not provide sufficient protection. Our patent position, like that of many biotechnology and pharmaceutical companies, is uncertain and involves complex legal and technical questions for which important legal principles are unresolved. We may not develop or obtain rights to products or processes that are patentable. Even if we do obtain patents, such patents may not adequately protect the technology we own or have licensed. In addition, others may challenge, seek to invalidate, infringe or circumvent any patents we own or license and rights we receive under those patents may not provide competitive advantages to us. For example, our European patent related to Agglomerated forms of Captisol was limited during an opposition proceeding and could be challenged further on appeal, and the rejection of our European patent application related to High Purity Captisol is currently being appealed.

We have obtained patent protection in the United States through 2025 on one or more Agglomerated forms of Captisol and through 2029 on one or more High Purity forms of Captisol. We also have filed patent applications covering the Captisol product that if issued, would not be set to expire until 2033 (for example, our patent WO 2013/130666, filed Feb. 27, 2013, contains composition of matter and use claims). There is no guarantee that our patents will be sufficient to prevent competitors from creating a generic form of Captisol and competing against us, or from developing combination patents for products that will prevent us from developing products using those APIs. In addition, most of the agreements in our Captisol outlicensing business, provide that once the relevant patent expires, the amount of royalties we receive will be reduced or eliminated.

Our collaborative partners may change their strategy or the focus of their development and commercialization efforts with respect to our partnered programs, and the success of our partnered programs could be adversely affected.

If our collaborative partners terminate their collaborations with us or do not commit sufficient resources to the development, manufacture, marketing or distribution of our partnered programs, we could be required to devote additional resources to our partnered programs, seek new collaborative partners or abandon such partnered programs, all of which could have an adverse effect on our business.

Third party intellectual property may prevent us or our partners from developing our potential products and we may owe a portion of any payments we receive from our collaborative partners to one or more third parties.

Our success will depend on our ability and the ability of our collaborative partners to avoid infringing the proprietary rights of others, both in the United States and in foreign countries. In addition, disputes with licensors under our license agreements may arise which could result in additional financial liability or loss of important technology and potential products and related revenue, if any. Further, the manufacture, use or sale of our potential products or our collaborative partners' products or potential products may infringe the patent rights of others. This could impact Captisol, Promacta, Kyprolis, Avinza, Duavee, Viviant and Conbriza, Nexterone, and other products or potential products.

Several drug companies and research and academic institutions have developed technologies, filed patent applications or received patents for technologies that may be related to our business. Others have filed patent applications and received patents that conflict with patents or patent applications we have licensed for our use, either by claiming the same methods or compounds or by claiming methods or compounds that could dominate those licensed to us. In addition, we may not be aware of all patents or patent applications that may impact our ability to make, use or sell any of our potential products. For example,



## Table of Contents

U.S. patent applications may be kept confidential while pending in the United States Patent and Trademark Office and patent applications filed in foreign countries are often first published six months or more after filing.

Disagreements or litigation with our collaborative partners could delay our ability and the ability of our collaborative partners to achieve milestones or our receipt of other payments. In addition, other possible disagreements or litigation could delay, interrupt or terminate the research, development and commercialization of certain potential products being developed by either our collaborative partners or by us. The occurrence of any of the foregoing problems could be time-consuming and expensive and could adversely affect our business.

Third parties have not directly threatened an action or claim against us, although we do periodically receive other communications or have other conversations with the owners of other patents or other intellectual property. If others obtain patents with conflicting claims, we may be required to obtain licenses to those patents or to develop or obtain alternative technology. We may not be able to obtain any such licenses on acceptable terms, or at all. Any failure to obtain such licenses could delay or prevent us from pursuing the development or commercialization of our potential products.

In general, litigation claims can be expensive and time consuming to bring or defend against and could result in settlements or damages that could significantly impact our results of operations and financial condition. We cannot predict or determine the outcome of these matters or reasonably estimate the amount or range of amounts of any fines or penalties that might result from a settlement or an adverse outcome. However, a settlement or an adverse outcome could have a material adverse effect on our financial position, liquidity and results of operations.

Although we have recently remediated a material weakness in our internal control over financial reporting, if we are unable to maintain the effectiveness of our internal controls, our financial results may not be accurately reported.

Management's assessment of the effectiveness of our internal control over financial reporting as of December 31, 2012 reported a material weakness in our internal control as a result of improper accounting for non-routine transactions and the controls over the determination of fair value of contingent liabilities, as described in our Annual Report on Form 10-K for the year ended December 31, 2012. We added a corporate controller to the finance and accounting staff to enhance our processes with the addition of a resource with the ability to research and understand the nuances of complex accounting standards. Additionally, we enhanced our controls over the determination of the fair value of contingent liabilities by including a formal review of mathematical calculations and completeness of such calculations. Although further and ongoing efforts will continue in 2014 and beyond to enhance our internal control over financial reporting, we believe that our remediation efforts now provide the foundation for compliance with the Committee of Sponsoring Organizations of the Treadway Commission (COSO) framework. As a result, our assessment of the effectiveness of our internal control over financial reporting as of December 31, 2013 no longer reports this material weakness or any other material weakness over financial reporting, and the audit report of our independent registered public accounting firm no longer expresses an adverse opinion on the effectiveness of our internal control over financial reporting as of December 31, 2013.

We may undertake strategic acquisitions in the future and any difficulties from integrating such acquisitions could adversely affect our stock price, operating results and results of operations.

We may acquire companies, businesses and products that complement or augment our existing business. We may not be able to integrate any acquired business successfully or operate any acquired business profitably. Integrating any newly acquired business could be expensive and time-consuming. Integration efforts often take a significant amount of time, place a significant strain on managerial, operational and financial resources and could prove to be more difficult or expensive than we predict. The diversion of our management's attention and any delay or difficulties encountered in connection with any future acquisitions we may consummate could result in the disruption of our

on-going business or inconsistencies in standards and controls that could negatively affect our ability to maintain third-party relationships. Moreover, we may need to raise additional funds through public or private debt or equity financing, or issue additional shares, to acquire any businesses or products, which may result in dilution for stockholders or the incurrence of indebtedness.

As part of our efforts to acquire companies, business or product candidates or to enter into other significant transactions, we conduct business, legal and financial due diligence with the goal of identifying and evaluating material risks involved in the transaction. Despite our efforts, we ultimately may be unsuccessful in ascertaining or evaluating all such risks and, as a result, might not realize the intended advantages of the transaction. If we fail to realize the expected benefits from acquisitions we may consummate in the future or have consummated in the past, whether as a result of unidentified risks, integration difficulties, regulatory setbacks, litigation with current or former employees and other events, our business, results of operations and financial condition could be adversely affected. If we acquire product candidates, we will also need to make certain assumptions about, among other things, development costs, the likelihood of receiving regulatory approval and the

## Table of Contents

market for such product candidates. Our assumptions may prove to be incorrect, which could cause us to fail to realize the anticipated benefits of these transactions.

In addition, we will likely experience significant charges to earnings in connection with our efforts, if any, to consummate acquisitions. For transactions that are ultimately not consummated, these charges may include fees and expenses for investment bankers, attorneys, accountants and other advisors in connection with our efforts. Even if our efforts are successful, we may incur, as part of a transaction, substantial charges for closure costs associated with elimination of duplicate operations and facilities and acquired In-Process Research and Development, or IPR&D, charges. In either case, the incurrence of these charges could adversely affect our results of operations for particular quarterly or annual periods.

We may not be able to hire and/or retain key employees.

If we are unable to hire and/or retain key employees, we may not have sufficient resources to successfully manage our assets or our business, and we may not be able to perform our obligations under various contracts and commitments. Furthermore, there can be no assurance that we will be able to retain all of our key management and scientific personnel. If we fail to retain such key employees, it could materially and adversely affect our business, financial condition, results of operations or the market price of our stock.

Aggregate revenues based on sales of our other products may not meet expectations.

Revenues based on sales of Avinza, Duavee, Conbriza and Nexterone may not meet expectations. Any setback that may occur with respect to these products could impair our operating results and/or reduce the market price of our stock. Setbacks for these products could include problems with shipping, distribution, manufacturing, product safety, marketing, government regulation or reimbursement, licenses and approvals, intellectual property rights, competition with existing or new products and physician or patient acceptance of the product, as well as higher than expected total rebates, returns or discounts. These products also are or may become subject to generic competition). Any such setback could reduce our revenue.

If plaintiffs bring product liability lawsuits against us or our partners, we or our partners may incur substantial liabilities and may be required to limit commercialization of our approved products and product candidates, and we may be subject to other liabilities related to the sale of our prior commercial product lines.

We and our partners face an inherent risk of product liability as a result of the clinical testing of our product candidates in clinical trials and face an even greater risk for commercialized products. Although we are not currently a party to product liability litigation, if we are sued, we may be held liable if any product or product candidate we develop causes injury or is found otherwise unsuitable during product testing, manufacturing, marketing or sale. Regardless of merit or eventual outcome, liability claims may result in decreased demand for any product candidates or products that we may develop, injury to our reputation, discontinuation of clinical trials, costs to defend litigation, substantial monetary awards to clinical trial participants or patients, loss of revenue and the inability to commercialize any products that we develop. We have product liability insurance that covers our clinical trials up to a \$5.0 million annual limit. If we are sued for any injury caused by our product candidates or any future products, our liability could exceed our total assets.

In addition, we agreed to indemnify Eisai and King Pharmaceuticals (now a subsidiary of Pfizer), under certain circumstances pursuant to the asset purchase agreements we entered into in connection with the sale of our prior commercial product lines. Some of our indemnification obligations still remain and our potential liability in certain circumstances is not limited to specific dollar amounts. We cannot predict the liabilities that may arise as a result of these matters. Any claims related to our indemnification obligations to Pfizer or Eisai could materially and adversely

affect our financial condition. In addition, Pfizer assumed our obligation to make payments to Organon based on net sales of Avinza (the fair value of which was \$11.7 million as of December 31, 2013). We remain liable to Organon in the event Pfizer defaults on this obligation. Any requirement to pay a material amount to Organon, could adversely affect our business and the price of our securities. The sale of our prior commercial product lines does not relieve us of exposure to product liability risks on products we sold prior to divesting these product lines. A successful product liability claim or series of claims brought against us may not be insured against and could result in payment of significant amounts of money and divert management's attention from our business.

## Table of Contents

If our partners do not reach the market with our partnered programs before our competitors offer products for the same or similar uses, or if our partners are not effective in marketing our partnered programs, our revenues from product sales, if any, will be reduced.

We face intense competition in our development activities. Our competitors might succeed in obtaining regulatory approval for competitive products more rapidly than our partners can for our partnered programs. In addition, competitors might develop technologies and products that are less expensive and perceived to be safer or more effective than those being developed by us or our partners, which could impair our product development and render our technology obsolete.

If our business does not perform according to our expectations, we may not be able to pay off our existing debt or have sufficient resources to operate our business as currently contemplated.

Our operations have consumed substantial amounts of cash since inception. As of December 31, 2013, we had negative working capital of \$4.1 million. In connection with our 2011 acquisition of CyDex, we entered into a \$20.0 million Loan and Security Agreement, or the Loan Agreement, with a lender. The loan was amended in January 2012 to increase the secured credit facility to \$27.5 million. The original \$20.0 million borrowed under the facility bears interest at a fixed rate of 8.6%. The additional \$7.5 million bears interest at a fixed rate of 8.9%. Under the terms of the secured debt, we made interest only payments through February 2013. Subsequent to the interest-only payments, the note amortizes with principal and interest payments through the remaining term of the loan. Additionally, we must also make an additional final payment equal to 6% of the total amount borrowed which is due at maturity and is being accreted over the life of the loan. The maturity date of the term loan is August 1, 2014. In March 2013, we prepaid \$7.0 million of the secured term loan credit facility. Additionally, we paid a prepayment fee of 1% of the prepayment amount, or \$0.1 million, and a prorated final-payment fee of 6% of the final payment or \$0.4 million. As of December 31, 2013, the remaining principal balance of the note was \$9.1 million.

In October 2013, we filed a universal shelf registration statement with the SEC that was automatically declared effective due to our status as a well-known seasoned issuer. This registration statement provides additional financial flexibility for us to sell shares of common stock or other equity or debt securities as needed at any time, including through our at-the-market equity issuance program. During the year ended December 31, 2013, we did not issue any common shares through this at-the market equity issuance program.

Our cash and cash equivalents as of December 31, 2013 was \$11.6 million. We believe that our capital resources, including our currently available cash, cash equivalents, and short-term investments as well as our current and future royalty revenues, will be adequate to fund our operations, including the repayment of our term loan which matures on August 1, 2014, at their current levels at least for the next 12 months. However, changes may occur that would cause us to consume available capital resources before that time and we may need to complete additional equity or debt financings to fund our operations. Our inability to obtain additional financing could adversely affect our business. Financings may not be available at all or on terms favorable to us. In addition, these financings, if completed, may not meet our capital needs and could result in substantial dilution to our stockholders. If adequate funds are not available, we may be required to delay, reduce the scope of or eliminate one or more of our research or drug development programs. We may also be required to liquidate our business or file for bankruptcy protection. Alternatively, we may be forced to attempt to continue development by entering into arrangements with collaborative partners or others that require us to relinquish some or all of our rights to technologies or drug candidates that we would not otherwise relinquish.

Our ability to use our net operating losses, or NOLs, to offset taxes that would otherwise be due could be limited or lost entirely.

Our ability to use our NOLs to offset taxes that would otherwise be due is dependent upon our generation of future taxable income before the expiration dates of the NOLs, and we cannot predict with certainty whether we will be able to generate future taxable income. In addition, even if we generate taxable income, realization of our NOLs to offset taxes that would otherwise be due could be restricted by annual limitations on use of NOLs triggered by a past or future “ownership change” under Section 382 of the Internal Revenue Code and similar state provisions. An “ownership change” may occur when there is a 50% or greater change in total ownership of our company by one or more 5% shareholders within a three-year period. The loss of some or all of our NOLs could materially and adversely affect our business, financial condition and results of operations. In addition, California and certain states have suspended use of NOLs for certain taxable years, and other states may consider similar measures. As a result, we may incur higher state income tax expense in the future. Depending on our future tax position, continued suspension of our ability to use NOLs in states in which we are subject to income tax could have an adverse impact on our operating results and financial condition. The calculation of the amount of our net operating loss

## Table of Contents

carryforwards may be changed as a result of a challenge by the IRS or other governmental authority or our learning of new information about the ownership of, and transactions in, our securities.

We use hazardous materials, which may expose us to significant liability.

In connection with our research and development activities, we handle hazardous materials, chemicals and various radioactive compounds. To properly dispose of these hazardous materials in compliance with environmental regulations, we are required to contract with third parties. We believe that we carry reasonably adequate insurance for toxic tort claims. However, we cannot eliminate the risk or predict the exposure of accidental contamination or injury from the handling and disposing of hazardous materials, whether by us or our third-party contractors. Any accident in the handling and disposing of hazardous materials may expose us to significant liability.

Our shareholder rights plan, concentration of ownership and charter documents may hinder or prevent change of control transactions.

Our shareholder rights plan and provisions contained in our certificate of incorporation and bylaws may discourage transactions involving an actual or potential change in our ownership. In addition, our Board of Directors may issue shares of common or preferred stock without any further action by the stockholders. Our directors and Biotechnology Value Fund, or BVF, own over 25% of our outstanding common stock as of December 31, 2013. BVF can increase its ownership level up to 24.99% under the terms of an agreement we have with BVF and BVF has agreed to vote 15% ownership in accordance with the Board's recommendations in the event that BVF exceeds a 19.99% ownership level. Such restrictions, circumstances and issuances may have the effect of delaying or preventing a change in our ownership. If changes in our ownership are discouraged, delayed or prevented, it would be more difficult for our current Board of Directors to be removed and replaced, even if you or our other stockholders believe that such actions are in the best interests of us and our stockholders.

Funding of our drug development programs may not result in future revenues.

Our drug development programs may require substantial additional capital to successfully complete them, arising from costs to: conduct research, preclinical testing and human studies; establish pilot scale and commercial scale manufacturing processes and facilities; and establish and develop quality control, regulatory, marketing, sales and administrative capabilities to support these programs. While we expect to fund our research and development activities from cash generated from royalties and milestones from our partners in various past and future collaborations to the extent possible, if we are unable to do so, we may need to complete additional equity or debt financings or seek other external means of financing. These financings could depress our stock price. If additional funds are required to support our operations and we are unable to obtain them on terms favorable to us, we may be required to cease or reduce further development or commercialization of our products, to sell some or all of our technology or assets or to merge with another entity.

Our results of operations and liquidity needs could be materially negatively affected by market fluctuations and economic downturn.

Our results of operations could be materially negatively affected by economic conditions generally, both in the United States and elsewhere around the world. Continuing concerns over inflation, energy costs, geopolitical issues, the availability and cost of credit, and the U.S. financial markets have contributed to increased volatility and diminished expectations for the economy and the markets going forward. These factors, combined with volatile oil prices, declining business and consumer confidence and increased unemployment, have precipitated an economic recession and fears of a possible depression. Domestic and international equity markets continue to experience heightened volatility and turmoil. These events and the continuing market upheavals may have an adverse effect on us. In the

event of a continuing market downturn, our results of operations could be adversely affected by those factors in many ways, including making it more difficult for us to raise funds if necessary, and our stock price may further decline. We cannot provide assurance that our investments are not subject to adverse changes in market value. If our investments experience adverse changes in market value, we may have less capital to fund our operations.



## Table of Contents

Our stock price has been volatile and could experience a sudden decline in value.

Our common stock has experienced significant price and volume fluctuations and may continue to experience volatility in the future. As a result, you may not be able to sell your shares quickly or at the latest market price if trading in our stock is not active or the volume is low. Many factors may have a significant impact on the market price of our common stock, including, but not limited to, the following factors: results of or delays in our preclinical studies and clinical trials; the success of our collaboration agreements; publicity regarding actual or potential medical results relating to products under development by us or others; announcements of technological innovations or new commercial products by us or others; developments in patent or other proprietary rights by us or others; comments or opinions by securities analysts or major stockholders; future sales of our common stock by existing stockholders; regulatory developments or changes in regulatory guidance; litigation or threats of litigation; economic and other external factors or other disaster or crises; the departure of any of our officers, directors or key employees; period-to-period fluctuations in financial results; and limited daily trading volume.

Impairment charges pertaining to goodwill, identifiable intangible assets or other long-lived assets from our mergers and acquisitions could have an adverse impact on our results of operations and the market value of our common stock.

The total purchase price pertaining to our acquisitions in recent years of Pharmacoepia, Neurogen, Metabasis and CyDex have been allocated to net tangible assets, identifiable intangible assets, in-process research and development and goodwill. To the extent the value of goodwill or identifiable intangible assets or other long-lived assets become impaired, we will be required to incur material charges relating to the impairment. Any impairment charges could have a material adverse impact on our results of operations and the market value of our common stock.

The occurrence of a catastrophic disaster could damage our facilities beyond insurance limits or we could lose key data which could cause us to curtail or cease operations.

We are vulnerable to damage and/or loss of vital data from natural disasters, such as earthquakes, tornadoes, power loss, fire, floods and similar events, as well as from accidental loss or destruction. If any disaster were to occur, our ability to operate our business could be seriously impaired. We have property, liability, and business interruption insurance which may not be adequate to cover our losses resulting from disasters or other similar significant business interruptions, and we do not plan to purchase additional insurance to cover such losses due to the cost of obtaining such coverage. Any significant losses that are not recoverable under our insurance policies could seriously impair our business, financial condition and prospects.

Item 1B. Unresolved Staff Comments  
None.

### Item 2. Properties

We currently occupy premises consisting of approximately 16,500 square feet of office and laboratory space in San Diego, leased through June 2019 which serves as our corporate headquarters. We believe this facility is adequate to meet our space requirements for the foreseeable future.

We lease approximately 1,500 square feet of laboratory space located at the Bioscience and Technology Business Center in Lawrence, Kansas, leased through December 2014.

We lease approximately 99,000 square feet in three facilities in Cranbury, New Jersey under leases that expire in 2016. We also sublease approximately 19,473 square feet of these facilities with subleases expiring in 2014 through 2016. We fully vacated these facilities in September 2010.

We also lease a 52,800 square foot facility in San Diego that is leased through July 2015. In January 2008, we began subleasing the 52,800 square foot facility under a sublease agreement through July 2015. We fully vacated this facility in February 2008.



Table of Contents

## Item 3. Legal Proceedings

From time to time we are subject to various lawsuits and claims with respect to matters arising out of the normal course of our business. Due to the uncertainty of the ultimate outcome of these matters, the impact on future financial results is not subject to reasonable estimates.

## Securities Litigation

On June 8, 2012, a federal securities class action and shareholder derivative lawsuit was filed in the Eastern District of Pennsylvania against Genaera Corporation and its officers, directors, major shareholders and trustee ("Genaera Defendants") for allegedly breaching their fiduciary duties to Genaera shareholders. The lawsuit also names us and our Chief Executive Officer John Higgins as additional defendants for allegedly aiding and abetting the Genaera Defendants' various breaches of fiduciary duties based on our purchase of a licensing interest in a development-stage pharmaceutical drug program from the Genaera Liquidating Trust in May 2010 and its subsequent sale of half of its interest in the transaction to Biotechnology Value Fund, Inc.

Following an amendment to the complaint and a round of motions to dismiss, the Court dismissed the amended complaint with prejudice on August 12, 2013. On September 10, 2013, the plaintiffs filed a notice of appeal.

According to the Third Circuit's briefing schedule, the plaintiffs opening brief is currently due on or before February 18, 2014, our answering brief is due thirty days later, and the plaintiff's reply brief, if any, is due fourteen days after that. We intend to continue to vigorously defend against the claims against us and Mr. Higgins in the lawsuit. Due to the complex nature of the legal and factual issues involved, however, the outcome of this matter is not presently determinable.

## Item 4. Mine Safety Disclosures

Not applicable.

## PART II

## Item 5. Market for Registrant's Common Equity, Related Stockholder Matters, and Issuer Purchases of Equity Securities

## Market Information

Our common stock is traded on the NASDAQ Global Market under the symbol "LGND."

The following table sets forth the high and low intraday sales prices for our common stock on the NASDAQ Global Market for the periods indicated:

	Price Range	
	High	Low
Year Ended December 31, 2013:		
1st Quarter	\$26.93	\$19.03
2nd Quarter	38.06	23.50
3rd Quarter	50.85	36.82
4th Quarter	58.48	43.20
Year Ended December 31, 2012:		
1st Quarter	\$18.74	\$11.44
2nd Quarter	17.27	11.21
3rd Quarter	19.85	15.80
4th Quarter	21.75	14.75

As of February 14, 2014, the closing price of our common stock on the NASDAQ Global Market was \$76.92.

## Holders

As of February 14, 2014, there were approximately 705 holders of record of the common stock.



Table of Contents

Performance Graph

The graph below shows the five-year cumulative total stockholder return assuming the investment of \$100 and is based on the returns of the component companies weighted monthly according to their market capitalizations. The graph compares total stockholder returns of our common stock, of all companies traded on the NASDAQ Stock market, as represented by the NASDAQ Composite® Index, and of the NASDAQ Biotechnology Stock Index, as prepared by The NASDAQ Stock Market Inc. The NASDAQ Biotechnology Stock Index tracks approximately 122 domestic biotechnology stocks.

The stockholder return shown on the graph below is not necessarily indicative of future performance and we will not make or endorse any predictions as to future stockholder returns.

	12/31/2008	12/31/2009	12/31/2010	12/31/2011	12/31/2012	12/31/2013
Ligand	100	% 79	% 54	% 72	% 126	% 320
NASDAQ Market (U.S. Companies)	100	% 145	% 172	% 170	% 201	% 281
NASDAQ Biotechnology Stocks	100	% 116	% 134	% 150	% 198	% 329

Table of Contents

## Item 6. Selected Consolidated Financial Data

The following selected historical consolidated financial and other data are qualified by reference to, and should be read in conjunction with, our consolidated financial statements and the related notes thereto appearing elsewhere herein and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.” Our selected statement of operations data set forth below for each of the years ended December 31, 2013, 2012, 2011, 2010, and 2009, and the balance sheet data as of December 31, 2013, 2012, 2011, 2010, and 2009, are derived from our consolidated financial statements.

	Year Ended December 31, (in thousands, except share data)				
	2013	2012	2011	2010	2009
Consolidated Statements of Operations Data:					
Royalties	\$23,584	\$14,073	\$9,213	\$7,279	\$8,334
Material sales	19,072	9,432	12,123	—	—
Collaborative research and development and other revenues	6,317	7,883	8,701	16,259	30,606
Total revenues	48,973	31,388	30,037	23,538	38,940
Cost of material sales	5,732	3,601	4,909	—	—
Research and development expenses	9,274	10,790	10,291	22,067	39,870
General and administrative expenses	17,984	15,782	14,583	12,829	15,211
Lease exit and termination costs	560	1,022	552	16,894	15,235
Write-off of acquired in-process research and development	480	—	2,282	2,754	442
Total operating costs and expenses	34,030	31,195	32,617	54,544	70,758
Accretion of deferred gain on sale leaseback	—	—	1,702	1,702	21,851
Income (loss) from operations	14,943	193	(878)	(29,304)	(9,967)
Income (loss) from continuing operations	8,832	(2,674)	9,712	(12,786)	(8,337)
Discontinued operations (1)	2,588	2,147	3	2,413	6,389
Net income (loss)	11,420	(527)	9,715	(10,373)	(1,948)
Basic per share amounts:					
Income (loss) from continuing operations	\$0.43	\$(0.14)	\$0.49	\$(0.65)	\$(0.44)
Discontinued operations (1)	0.13	0.11	—	0.12	0.34
Net income (loss)	\$0.56	\$(0.03)	\$0.49	\$(0.53)	\$(0.10)
Weighted average number of common shares-basic	20,312,395	19,853,095	19,655,632	19,613,201	18,862,751
Diluted per share amounts:					
Income (loss) from continuing operations	\$0.43	\$(0.14)	\$0.49	\$(0.65)	\$(0.44)
Discontinued operations (1)	0.12	0.11	—	0.12	0.34
Net income (loss)	\$0.55	\$(0.03)	\$0.49	\$(0.53)	\$(0.10)
Weighted average number of common shares-diluted	20,745,454	19,853,095	19,713,320	19,613,201	18,862,751

Table of Contents

	December 31,				
	2013	2012	2011	2010	2009
	(in thousands)				
Consolidated Balance Sheet Data:					
Cash, cash equivalents, short-term investments and restricted cash and investments	\$ 17,320	\$ 15,148	\$ 18,382	\$ 24,038	\$ 54,694
Working capital	(4,058	)(11,616	)(11,413	) 3,531	15,994
Total assets	104,713	104,260	120,583	75,559	141,807
Current portion of deferred revenue, net	116	486	1,240	—	4,989
Current portion of deferred gain	—	—	—	1,702	1,702
Long-term obligations (excludes long-term portions of deferred revenue, net and deferred gain)	24,076	39,967	56,945	36,030	72,350
Long-term portion of deferred revenue, net	2,085	2,369	3,466	2,546	3,495
Long-term portion of deferred gain	—	—	—	—	1,702
Common stock subject to conditional redemption	—	—	8,344	8,344	8,344
Accumulated deficit	(671,339	)(682,759	)(682,232	)(691,947	)(681,574
Total stockholders' equity (deficit)	49,613	26,485	8,185	(4,849	) 3,744

We sold our Oncology product line ("Oncology") on October 25, 2006 and we sold our Avinza product line ("Avinza") (1) on February 26, 2007. The operating results for the Oncology and Avinza product lines have been presented in our consolidated statements of operations as "Discontinued Operations."

#### Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

Caution: This discussion and analysis may contain predictions, estimates and other forward-looking statements that involve a number of risks and uncertainties, including those discussed in "Item 1A. Risk Factors." This outlook represents our current judgment on the future direction of our business. These statements include those related to our Captisol related revenue, our Promacta, Kyprolis, and other product royalty revenues, product returns, and product development. Actual events or results may differ materially from our expectations. For example, there can be no assurance that our revenues or expenses will meet any expectations or follow any trends, that we will be able to retain our key employees or that we will be able to enter into any strategic partnerships or other transactions. We cannot assure you that we will receive expected Promacta, Kyprolis, Captisol and other product revenues to support our ongoing business or that our internal or partnered pipeline products will progress in their development, gain marketing approval or achieve success in the market. In addition, ongoing or future arbitration, or litigation or disputes with third parties may have a material adverse effect on us. Such risks and uncertainties, and others, could cause actual results to differ materially from any future performance suggested. We undertake no obligation to make any revisions to these forward-looking statements to reflect events or circumstances arising after the date of this annual report. This caution is made under the safe harbor provisions of Section 21E of the Securities Exchange Act of 1934, as amended.

Our trademarks, trade names and service marks referenced herein include Ligand. Each other trademark, trade name or service mark appearing in this annual report belongs to its owner.

References to "Ligand Pharmaceuticals Incorporated," "Ligand," the "Company," "we" or "our" include our wholly owned subsidiaries—Ligand JVR, Allergan Ligand Retinoid Therapeutics, Seragen, Inc., Pharmacopeia, LLC, or Pharmacopeia, Neurogen Corporation, or Neurogen, CyDex Pharmaceuticals, Inc., or CyDex, Metabasis Therapeutics, or Metabasis, and Nexus Equity VI LLC.

## Table of Contents

We are a biotechnology company that operates with a business model focused on developing or acquiring revenue generating assets and coupling them with a lean corporate cost structure. Our goal is to create a sustainably profitable business and generate meaningful value for our stockholders. Since a portion of our business model is based on the goal of partnering with other pharmaceutical companies to commercialize and market our assets, a significant amount of our revenue is based largely on payments made to us by partners for royalties, milestones and license fees. We recognized the important role of the drug reformulation segment in the pharmaceutical industry and in 2011 added Captisol to our technology portfolio. Captisol is a formulation technology that has enabled six FDA approved products, including Onyx's Kyprolis and Baxter International's Nexterone and is currently being developed in a number of clinical-stage partner programs. In comparison to our peers, we believe we have assembled one of the largest and most diversified asset portfolios in the industry with the potential to generate significant revenue in the future. The therapies in our development portfolio address the unmet medical needs of patients for a broad spectrum of diseases including hepatitis, muscle wasting, multiple myeloma, Alzheimer's disease, dyslipidemia, diabetes, anemia, epilepsy, FSGS and osteoporosis. We have established multiple alliances with the world's leading pharmaceutical companies including GlaxoSmithKline, Onyx Pharmaceuticals (a subsidiary of Amgen, Inc.), Merck, Pfizer, Baxter International, Lundbeck Inc., Eli Lilly and Co., and Spectrum Pharmaceuticals, Inc.

In December 2012, we received a milestone payment of 620,000 shares of common stock in partner Retrophin, Inc. or Retrophin. The milestone arose under the previously executed license agreement for the development and commercialization of Retrophin's lead clinical candidate, Sparsentan, and was triggered by the completion of Retrophin's merger with Desert Gateway, Inc. and its transition to a publicly traded company. We recorded milestone revenue of \$1.2 million, net of amounts owed to a third party. The fair value of the shares received was determined by an independent valuation firm. The shares issued to us represent approximately 3.4% and 6.9% of Retrophin's outstanding capital stock as of December 31, 2013 and 2012, respectively, and were subject to a one-year trading restriction which lifted in December 2013. Additionally, in early 2013 we received a \$1.4 million time based milestone payment from Retrophin and remitted \$0.2 million to former license holders under the terms of a previous license agreement for Sparsentan.

In March 2013, we entered into a License Agreement with Spectrum Pharmaceuticals, Inc. or Spectrum. Under the License Agreement, we granted to Spectrum an exclusive, nontransferable, worldwide license to such intellectual property rights that will enable Spectrum to develop and potentially commercialize Captisol-enabled propylene glycol-free melphalan. Contemporaneously with the entry into the license agreement, we entered into a supply agreement to provide Captisol to Spectrum. Under the Supply Agreement, Spectrum agreed to purchase its Captisol requirements for the development of the compound contemplated by the license agreement, as well as any Captisol required for any product that is successfully commercialized. In connection with this license we received a non-refundable license issuance fee of \$3 million. Additionally, we are entitled to milestone payments and royalties on future net sales of the Captisol-enabled melphalan product. This program is currently enrolling patients in a pivotal clinical trial.

In April 2013, we entered into a Royalty Stream and Milestone Payments Purchase Agreement with Selexis SA or Selexis, to acquire a portfolio of possible future royalty and milestone payment rights based on over 15 Selexis commercial license agreement programs with various pharmaceutical-company counterparties. In return, we paid Selexis an upfront payment of \$3.5 million, and expect to make an additional \$1 million cash payment on the first anniversary of the closing.

In May 2013, by virtue of ARES Trading SA (a unit of Merck KGaA) not having exercised its option to obtain a further related license from us, the Research License and Option Agreement we and ARES Trading SA had entered into in April 2012 terminated in accordance with its terms, and the rights to an anti-inflammatory discovery research program that we had licensed to ARES Trading SA under this agreement reverted to us.

In May 2013, our partner Melinta Therapeutics, Inc. (formerly Rib-X) announced the initiation of a Phase 3 clinical trial of Captisol-enabled intravenous formulation of delafloxacin for the first-line treatment of acute bacterial skin and skin structure infections (ABSSSI), including infections caused by MRSA. Under the terms of a license and supply



agreement, we earned a \$0.5 million milestone payment.

In July 2013, we entered into a global license agreement with Azure Biotech for the development of a novel formulation of lasofoxifene. Under the terms of the agreement, we are entitled to receive \$2.6 million in potential development and regulatory milestones and a 5% royalty on future net sales. Under this agreement, we retain the rights to the oral formulation originally developed by Pfizer. Additionally, in July 2013, we entered into a license agreement with Ethicor Pharma Ltd. for the manufacture and distribution of the oral formulation of lasofoxifene in the European Economic Area, Switzerland and the Indian Subcontinent. Under the terms of the agreement, we are entitled to receive potential sales milestones of up to \$16 million and a royalty of 25% on future net sales.

## Table of Contents

In July 2013, the FDA granted orphan-drug designation for our proprietary Captisol-enabled Topiramate Injection for the treatment of partial onset or primary generalized tonic-clonic seizures in hospitalized epilepsy patients who are unable to take oral topiramate. In August 2013, we entered a global license agreement with CURx Pharmaceuticals, Inc. for the development and commercialization of Topiramate and earned a milestone payment of \$0.2 million for the orphan-drug designation.

In July 2013, we and The Medicines Company, or MedCo, mutually terminated the License Agreement dated June 1, 2011 and the related Supply Agreement dated June 1, 2011. These agreements were with our subsidiary CyDex and related to the development of Captisol-enabled intravenous Clopidogrel. Upon termination, the licensed rights relating to the compound were returned to us. MedCo recently conducted a pharmacokinetic and pharmacodynamic study of oral Clopidogrel and Captisol-enabled intravenous Clopidogrel in healthy volunteers. The study indicated a potential difference in metabolism between the oral and intravenous routes of administration for Clopidogrel, and MedCo elected not to proceed with further development.

In July 2013, Merck notified us that it has discontinued clinical development of dinaciclib for chronic lymphocytic leukemia.

In August 2013, we entered a Commercial License Agreement with Sage Therapeutics Inc. This agreement replaces a prior agreement between Sage Therapeutics and our subsidiary CyDex. In October 2011, Sage originally obtained an exclusive right to use Captisol® in SAGE's development and commercialization of therapeutic drugs formulating certain allosteric receptor modulators with Captisol against identified central nervous system disorders. Sage exercised certain product commercialization options in December 2012 and then replaced that agreement with the Commercial License Agreement in August 2013. Upon commercialization, we could potentially receive milestone payments of \$4.5 million for Captisol-enabled programs, plus royalties of 3% on net sales for products that use the Captisol technology. Additionally, we could receive commercial revenue from the shipment of Captisol to Sage for clinical and commercial activities.

In October 2013, our partner, Pfizer received approval from the FDA for Duavee, for the treatment of moderate-to-severe vasomotor symptoms (VMS) associated with menopause and the prevention of postmenopausal osteoporosis. We earned a \$0.4 million milestone payment for the approval.

In October 2013, the FDA accepted our Investigational New Drug, or IND, application for our proprietary Glucagon receptor antagonist product (LGD-6972) candidate for the treatment of diabetes. LGD-6972 was acquired in connection with our acquisition of Metabasis and we may be required to remit payment to the contingent value right, or CVR, holders upon the sale or partnering of the asset. We initiated a Phase 1 clinical trial in the fourth quarter of 2013.

In November 2013, our partner, Merck submitted an NDA for Captisol-enabled Noxafil-IV. Merck is currently conducting a pivotal study for this program and it filed a 505(b)(2) application in 2013 for approval in the United States and European Union to market its Captisol program. In the United States, the New Drug Application, or NDA, for Noxafil-IV was filed and received FDA Priority Review in November 2013. We earned a \$0.2 million milestone for submission of the NDA.

## Results of Operations

Total revenues for 2013 were \$49.0 million compared to \$31.4 million in 2012 and \$30.0 million in 2011. Our income from continuing operations for 2013 was \$8.8 million or \$0.43 per diluted share, compared to a loss from continuing operations of \$2.7 million in 2012, or \$0.14 per diluted share, and income from continuing operations of \$9.7 million, or \$0.49 per diluted share, in 2011.

## Royalty Revenue

Royalty revenues were \$23.6 million in 2013, compared to \$14.1 million in 2012 and \$9.2 million in 2011. The increase in royalty revenue of \$9.5 million and \$4.9 million for the year ended December 31, 2013 and 2012, respectively is primarily due to an increase in Promacta and Kyprolis royalties.

Table of Contents

## Material Sales

We recorded material sales of Captisol of \$19.1 million in 2013 compared to \$9.4 million in 2012 and \$12.1 million in 2011. The increase in material sales of \$9.7 million for the year ended December 31, 2013 compared to 2012 is due to timing of customer purchases of Captisol as well as an increase in customer purchases for use in clinical trials which has a higher gross margin. The decrease in material sales of \$2.7 million for the year ended December 31, 2012 compared to 2011 is due to timing of customer purchases of Captisol.

## Collaborative Research and Development and Other Revenue

We recorded collaborative research and development and other revenues of \$6.3 million in 2013 compared to \$7.9 million in 2012 and \$8.7 million in 2011. The decrease of \$1.6 million for the year ended December 31, 2013, compared to the same period in 2012 is due to timing of achievement of certain regulatory milestones and licensing payments for the year ended December 31, 2013 compared with the same period in 2012. The decrease in collaborative research and development and other revenue of \$0.8 million for the year ended December 31, 2012, compared to 2011 is primarily due to the recognition of \$1.3 million of deferred revenue related to the previous sale of royalty rights for the year ended December 31, 2011, partially offset by an increase in license fees and milestones of \$0.5 million for the year ended December 31, 2012.

## Cost of material sales

Cost of sales were \$5.7 million in 2013 compared to \$3.6 million in 2012 and \$4.9 million in 2011. The increase of \$2.1 million for the year ended December 31, 2013, compared to the same period in 2012 is due to timing of customer purchases of Captisol as well as an increase in purchases for use in clinical trials. The decrease of \$1.3 million, for the year ended December 31, 2012, compared to 2011 is due to the decrease in material sales of Captisol.

## Research and Development Expenses

Research and development expenses for 2013 were \$9.3 million compared to \$10.8 million in 2012 and \$10.3 million in 2011. The decrease of \$1.5 million is primarily due to the timing of costs associated with internal programs. The increase in research and development expenses of \$0.5 million for the year ended December 31, 2012 compared to 2011 is primarily due to timing of costs associated with internal programs.

As summarized in the table below, we are developing several proprietary products for a variety of indications. Our programs are not limited to the following, but are representative of a range of future licensing opportunities to expand our partnered asset portfolio.

Program	Disease/Indication	Development Phase
HepDirect	Liver Diseases	Preclinical
Oral Human Granulocyte Colony Stimulating Factor	Neutropenia	Preclinical
IRAK-4	Inflammation	Preclinical
Glucagon Receptor Antagonist	Diabetes	Phase 1
Selective Androgen Receptor Modulator	Various	Phase 2-ready
Captisol-Enabled Clopidogrel	Anti-coagulant	Phase 3

We do not provide forward-looking estimates of costs and time to complete our ongoing research and development projects as such estimates would involve a high degree of uncertainty. Uncertainties include our inability to predict the outcome of complex research, our inability to predict the results of clinical studies, regulatory requirements placed

upon us by regulatory authorities such as the FDA and EMA, our inability to predict the decisions of our collaborative partners, our ability to fund research and development programs, competition from other entities of which we may become aware in future periods, predictions of market potential from products that may be derived from our research and development efforts, and our ability to recruit and retain personnel or third-party research organizations with the necessary knowledge and skills to perform certain research. Refer to “Item 1A. Risk Factors” for additional discussion of the uncertainties surrounding our research and development initiatives.

## Table of Contents

### General and Administrative Expenses

General and administrative expenses were \$18.0 million for the year ended December 31, 2013 compared to \$15.8 million for 2012 and \$14.6 million for 2011. The increase in general and administrative expenses for the year ended December 31, 2013 compared with 2012 of \$2.2 million is primarily due to an increase in non-cash stock-based compensation and patent and other legal expenses in 2013. The increase in expenses for the year ended December 31, 2012 compared with 2011 is primarily due to an increase in tax consulting project-related expenses and legal expenses compared to the prior year.

### Lease Exit and Termination Costs

For the years ended December 31, 2013 and 2012, we had lease exit obligations of \$5.9 million and \$9.0 million, respectively. The lease exit obligations are related to facilities in San Diego, California and Cranbury, New Jersey. The San Diego facility is under an operating lease through July 2015. We fully vacated this facility in February 2008 and sublet it through the term of our lease. Additionally, we ceased use of our facility located in Cranbury, New Jersey in September 2010. The remaining lease obligations run through August 2016, a portion of which is subleased with subleases expiring between August 2014 and 2016. We recorded lease exit and termination costs of \$0.6 million for the year ended December 31, 2013, compared to \$1.0 million for 2012, and \$0.6 million in 2011. Lease exit and termination costs for the years ended December 31, 2013, 2012, and 2011 consisted of accretion costs and adjustments to the liability for lease exit costs due to changes in leasing assumptions.

### Write-off of in-process research and development

For the year ended December 31, 2013, we recorded a non-cash impairment charge of \$0.5 million for the write-off of in-process research and development for Captisol-enabled intravenous Clopidogrel. Captisol-enabled intravenous Clopidogrel is an intravenous formulation of the anti-platelet medication designed for situations where the administration of oral platelet inhibitors is not feasible or desirable. For the year ended December 31, 2012, there was no write-off of in-process research and development recorded. In 2011, we recorded a non-cash impairment charge of \$1.1 million for the write-off of intellectual property and interests in future milestones and royalties for MEDI-528, an IL-9 antibody program by AstraZeneca's subsidiary, MedImmune. The asset was impaired upon receipt of notice from MedImmune that it was exercising its right to terminate the collaboration and license agreement. Additionally, in 2011, we recorded a non-cash impairment charge of \$1.2 million for the write-off of interests in future milestones for TRPV1, a collaborative research and licensing program between us and Merck, related to the physiology, pharmacology, chemistry and potential therapeutic applications and potential clinical utilities related to Vanilloid Receptors, subtype 1. The asset was impaired upon receipt of notice from Merck in October 2011 that it was exercising its right to terminate the collaboration and license agreement.

### Accretion of Deferred Gain on Sale Leaseback

In 2006, we entered into an agreement for the sale of our real property located in San Diego, California for a purchase price of \$47.6 million. This property, with a net book value of \$14.5 million, included one building totaling approximately 82,500 square feet, the land on which the building is situated, and two adjacent vacant lots. As part of the sale transaction, we agreed to lease back the building for a period of 15 years. We recognized an immediate pre-tax gain on the sale transaction of \$3.1 million in 2006 and deferred a gain of \$29.5 million on the sale of the building. The deferred gain was being recognized as an offset to operating expense on a straight-line basis over the 15 year term of the lease at a rate of approximately \$2.0 million per year. In 2009, we entered into a lease termination agreement for this building. As a result, we recognized an additional \$20.4 million of accretion of deferred gain during the quarter ended September 30, 2009, and recognized the remaining balance of the deferred gain of \$3.1 million through the term of our new building lease, which expired in December 2011. The amount of the deferred gain

recognized for the year ended December 31, 2011 was \$1.7 million. The deferred gain was fully amortized as of December 31, 2011.

## Table of Contents

### Interest expense, net

Interest expense was \$2.1 million for the year ended December 31, 2013 compared to \$2.9 million in 2012 and \$2.3 million in 2011. The decrease in interest expense of \$0.8 million for the year ended December 31, 2013 compared with 2012 is due to a lower principal balance related to our \$7.0 million prepayment of principal in March 2013 as well as scheduled principal amortization from March 2013 through December 2013. The increase in interest expense for the year ended December 31, 2012 compared to 2011 was due to the increase in the outstanding balance of notes payable at December 31, 2012 compared to 2011. Additionally, the \$20 million loan obtained to acquire CyDex in January 2011 was outstanding for a partial period for the year ended December 31, 2011.

### Change in Contingent Liabilities

We recorded an increase in contingent liabilities of \$3.6 million for the year ended December 31, 2013 compared to \$1.7 million in 2012 and \$1.0 million in 2011. The increase in contingent liabilities for the year ended December 31, 2013 is due primarily to the increase in the Metabasis CVR liability of \$4.2 million. The Metabasis CVR liability is marked-to-market at each reporting period based upon the quoted market prices of the underlying CVR. This was partially offset by a decrease of \$0.6 million in CyDex contingent liabilities, primarily due to a decrease in amounts potentially due to CyDex CVR holders and former license holders related to Captisol-enabled Clopidogrel, partially offset by an increase in the revenue-sharing CVR liability to former CyDex shareholders. The increase in contingent liabilities for the year ended December 31, 2012 is due to increases in amounts owed to CyDex CVR holders and former license holders of \$3.4 million, partially offset by decreases in amounts owed to Metabasis and Neurogen shareholders of \$1.1 million and \$0.7 million, respectively. The increase in contingent liabilities for the year ended December 31, 2011 is due to an increase in amounts potentially due to Metabasis shareholders of \$1.1 million, partially offset by a decrease in amounts owed to CyDex CVR holders and former license holders of \$0.1 million.

### Other, net

We recorded other expense of \$0.1 million for the year ended December 31, 2013 compared to other income of \$0.5 million in 2012 and \$0.6 million in 2011. Other expense for 2013 is primarily due to an increase in amounts owed to sublicensees, partially offset by changes in certain liabilities. Other income for 2012 is primarily due to changes in certain liabilities. Other income for 2011 primarily relates to income related to the gain on the sale of property and equipment and decreases in certain liabilities.

### Income Taxes

We recorded income tax expense from continuing operations of \$0.4 million for the year ended December 31, 2013 compared to an income tax benefit from continuing operations of \$1.2 million for the year ended December 31, 2012 and an income tax benefit of \$13.3 million for the year ended December 31, 2011. The income tax expense recognized in 2013 is primarily attributable to deferred taxes associated with the amortization of acquired IPR&D assets for tax purposes. The income tax benefit in 2012 is principally due to a requirement under Accounting Standards Codification ("ASC"), 740, Accounting for Income Taxes, that a Company to consider all sources of income in order to determine the tax benefit resulting from a loss from continuing operations. As a result of the requirement under ASC 740-20-45-7, the pretax income which we generated from discontinued operations was a source of income which resulted in the partial realization of the current year loss from continuing operations. Thus, we recorded an approximate \$1.5 million tax benefit to continuing operations and an offsetting \$1.5 million charge to discontinued operations. In addition, we realized a tax benefit as a result of California voters approving legislation in November 2012 which required a single sales factor income apportionment methodology beginning in 2013 and resulted in a decrease in our future California deferred income tax obligations. The income tax benefit in 2011 was principally the result of net deferred tax liabilities recorded in connection with our acquisition of Cydex. The net



deferred tax liabilities assumed in the Cydex acquisition became a future source of income to support the realization of deferred tax assets and resulted in the release of a portion of our valuation allowance against deferred tax assets.

## Table of Contents

### Discontinued Operations, net

#### Avinza Product Line

On September 6, 2006, we and King Pharmaceuticals, now a subsidiary of Pfizer, entered into a purchase agreement, or the Avinza Purchase Agreement, pursuant to which Pfizer acquired all of our rights in and to Avinza in the United States, its territories and Canada, including, among other things, all Avinza inventory, records and related intellectual property, and to assume certain liabilities as set forth in the Avinza Purchase Agreement.

Pursuant to the terms of the Avinza Purchase Agreement, we retained the liability for returns of product from wholesalers that had been sold by us prior to the close of this transaction. Accordingly, as part of the accounting for the gain on the sale of Avinza, we recorded a reserve for Avinza product returns. For the years ended December 31, 2013, 2012, and 2011, we recognized pre-tax gains of \$2.6 million, \$3.7 million and \$0, respectively, due to subsequent changes in certain estimates of assets and liabilities recorded as of the sale date.

#### Income tax expense on discontinued operations

In 2012, we recorded income tax expense on discontinued operations of \$1.5 million (please see the discussion on income taxes above). There was no income tax expense on discontinued operations for the years ended December 31, 2013 and 2011.

#### Liquidity and Capital Resources

We have financed our operations through offerings of our equity securities, borrowings from long-term debt, issuance of convertible notes, product sales and the subsequent sales of our commercial assets, royalties, collaborative research and development and other revenues, capital and operating lease transactions.

We have incurred significant losses since inception. At December 31, 2013, our accumulated deficit was \$671.3 million and we had negative working capital of \$4.1 million. We believe that cash flows from operations will improve due to consistent Captisol sales, an increase in royalty revenues driven primarily from continued increases in Promacta and Kyprolis sales, recent product approvals and regulatory developments, as well as anticipated new license and milestone revenues. In the event revenues and operating cash flows do not meet expectations, management plans to reduce discretionary expenses. However, it is possible that we may be required to seek additional financing. There can be no assurance that additional financing will be available on terms acceptable to management, or at all. We believe our available cash, cash equivalents, and short-term investments as well as our current and future royalty, license and milestone revenues will be sufficient to satisfy our anticipated operating and capital requirements, through at least the next twelve months. Our future operating and capital requirements will depend on many factors, including, but not limited to: the pace of scientific progress in our research and development programs; the potential success of these programs; the scope and results of preclinical testing and clinical trials; the time and costs involved in obtaining regulatory approvals; the costs involved in preparing, filing, prosecuting, maintaining and enforcing patent claims; competing technological and market developments; the amount of royalties on sales of the commercial products of our partners; the efforts of our collaborative partners; obligations under our operating lease agreements; and the capital requirements of any companies we acquire, including Pharmacopeia, Neurogen, Metabasis and CyDex. We believe that the actions presently being taken to generate sufficient operating cash flow provide the opportunity for us to continue as a going concern. While we believe in the viability of our strategy to generate sufficient operating cash flow and in our ability to raise additional funds, there can be no assurances to that effect. Our ability to achieve our operational targets is dependent upon our ability to further implement our business plan and generate sufficient operating cash flow.

In January 2011, we entered into a \$20 million secured term loan credit facility with Oxford Financial Group. The loan was amended in January 2012 to increase the secured credit facility to \$27.5 million. The original \$20 million

borrowed under the facility bears interest at a fixed rate of 8.6%. The additional \$7.5 million bears interest at a fixed rate of 8.9%. Under the terms of the secured debt, we made interest-only payments through February 2013. Subsequent to the interest-only payments, the note amortizes with principal and interest payments through the remaining term of the loan. Additionally, we must also make an additional final payment equal to 6% of the total amount borrowed which is due at maturity and is being accreted over the life of the loan. The maturity date of the term loan is August 1, 2014.

In March 2013, we prepaid \$7.0 million of the secured term loan credit facility. Additionally, we paid a prepayment fee of 1% of the prepayment amount, or \$0.1 million and a prorated final-payment fee of 6% of the final payment, or \$0.4 million. As of December 31, 2013, the remaining principal balance of the note was \$9.1 million.

## Table of Contents

In October 2013, we filed a universal shelf registration statement with the SEC that was automatically declared effective due to our status as a well-known seasoned issuer. This registration statement provides additional financial flexibility for us to sell shares of common stock or other equity or debt securities as needed at any time, including through our at-the-market equity issuance program. During the year ended December 31, 2013, we did not issue any common shares through this at-the market equity issuance program.

In connection with the acquisition of CyDex on January 24, 2011, we issued a series of CVR agreements and assumed certain contractual obligations. We paid the CVR holders \$4.3 million in January 2012 and may be required to pay additional amounts upon achievement of certain clinical and regulatory milestones to the CyDex CVR holders and former license holders. In 2011, \$0.9 million was paid to the CyDex shareholders upon completion of a licensing agreement with MedCo for the Captisol enabled intravenous formulation of Clopidogrel. An additional \$2 million was paid to the CyDex shareholders upon acceptance by the FDA of the New Drug Application submitted by Onyx and an additional \$3.5 million was paid upon approval by the FDA of Kyprolis for the potential treatment of patients with relapsed and refractory multiple myeloma. In addition, we agreed to pay CyDex shareholders, for each respective year from 2011 through 2016, (i) 20% of all CyDex-related revenue, but only to the extent that and beginning only when CyDex-related revenue for such year exceeds \$15.0 million, plus (ii) an additional 10% of all CyDex-related revenue recognized during such year, but only to the extent that and beginning only when aggregate CyDex-related revenue for such year exceeds \$35.0 million. We paid \$0.3 million to the CyDex shareholders in March 2012 for 20% of all 2011 CyDex-related revenue in excess of \$15 million. For the year ended December 31, 2012, CyDex related revenue did not exceed \$15 million. The revenue sharing payment for the year ended December 31, 2013 was \$2.5 million, \$0.9 million of which was paid during 2013.

We are also required by the CyDex CVR Agreement to dedicate at least five experienced full-time employee equivalents per year to the acquired business and to invest at least \$1.5 million per year, inclusive of such employee expenses, in the acquired business, through 2015. We have exceeded our commitment for the year ending December 31, 2013.

### Operating Activities

Operating activities provided cash of \$20.7 million and \$0.2 million in 2013 and 2012, respectively and used cash of \$1.2 million in 2011.

The cash provided in 2013 reflects net income of \$11.4 million, adjusted by \$2.6 million of gain from discontinued operations and \$13.2 million of non-cash items to reconcile the net income to net cash used in operations. These reconciling items primarily reflect a non-cash change in estimated value of contingent liabilities of \$3.6 million, depreciation and amortization of \$2.7 million, stock-based compensation of \$5.7 million, write-off of in-process research and development \$0.5 million, accretion of notes payable of \$0.4 million, and net deferred tax assets and liabilities of \$0.4 million. The cash provided by operations in 2013 is further impacted by changes in operating assets and liabilities due primarily to a decrease in accounts receivable of \$2.4 million, a decrease in inventory of \$0.6 million, and a decrease in other assets of \$0.1 million. Partially offsetting this, accounts payable and accrued liabilities decreased \$2.8 million, other liabilities decreased \$0.4 million and deferred revenue decreased \$0.7 million. Net cash used in operating activities of discontinued operations was \$0.6 million in 2013.

The cash provided in 2012 reflects a net loss of \$0.5 million, adjusted by \$2.1 million of gain from discontinued operations and \$6.5 million of non-cash items to reconcile the net income to net cash used in operations. These reconciling items primarily reflect a non-cash change in estimated value of contingent liabilities of \$1.7 million, depreciation and amortization of \$2.7 million, stock-based compensation of \$4.1 million and other changes of \$0.5 million, partially offset by an increase in net deferred tax assets and liabilities of \$1.2 million, and receipt of a non-cash milestone of \$1.2 million. The cash provided by operations in 2012 is further impacted by changes in operating assets and liabilities due primarily to a decrease in accounts receivable of \$1.5 million, a decrease in inventory of \$1.0 million, a decrease in other current assets of \$0.5 million, a decrease in other long term assets of \$0.3 million, and an increase in other liabilities of \$0.5 million. Partially offsetting this, accounts payable and accrued liabilities decreased \$4.8 million and deferred revenue decreased \$1.9 million. Net cash used in operating activities of

discontinued operations was \$0.9 million in 2012.

The use of cash in 2011 reflects net income of \$9.7 million, adjusted by \$5.0 million of non-cash items to reconcile the net income to net cash used in operations. These reconciling items primarily reflect deferred income taxes of \$13.4 million, accretion of deferred gain on sale leaseback transaction of \$1.7 million and a gain on asset write-offs of \$0.5 million, partially offset by a non-cash change in estimated value of contingent liabilities of \$1.9 million, a write-off of acquired in-process research and development of \$2.3 million, depreciation and amortization of \$2.8 million, and stock-based compensation of \$3.4 million. The use of cash in 2011 is further impacted by changes in operating assets and liabilities due primarily to an increase in accounts receivable of \$3.9 million and a decrease in accounts payable and accrued liabilities of \$11.6 million, partially offset by an increase in other current assets of \$5.5 million, an increase in inventory of \$1.1 million, a decrease in deferred revenue of

## Table of Contents

\$2.2 million, and a decrease in other liabilities of \$0.9 million. None of the cash used in operating activities for 2011 related to discontinued operations.

### Investing Activities

Investing activities used cash of \$5.0 million in 2013, provided by cash of \$1.3 million in 2012, and used cash of \$25.2 million in 2011.

Cash used by investing activities in 2013 primarily reflects the purchase of commercial license rights of \$3.6 million, payments to CyDex CVR holders of \$1.0 million, and purchases of property, building and equipment of \$0.4 million. None of the cash provided by investing activities for 2013 related to discontinued operations.

Cash used by investing activities in 2012 primarily reflects payments to CyDex CVR holders of \$8.0 million and purchases of property, building and equipment of \$0.6 million, partially offset by proceeds from the sale of short-term investments of \$10.0 million. None of the cash provided by investing activities for 2012 related to discontinued operations.

Cash used by investing activities in 2011 primarily reflects cash used for the acquisition of CyDex of \$32.0 million, payments made to CyDex CVR holders of \$2.9 million, and purchases of short term investments of \$10.0 million, partially offset by proceeds from the sale of short-term investments of \$19.3 million and proceeds from the sale of property and equipment of \$0.5 million. None of the cash provided by investing activities for 2011 related to discontinued operations.

### Financing Activities

Financing activities used cash of \$16.5 million in 2013 and provided cash of \$3.9 million in 2012 and \$30.0 million in 2011.

Cash used in financing activities in 2013 primarily reflects the repayment of debt of \$19.6 million, partially offset by proceeds of \$3.1 million received from stock option exercises and purchases under the employee stock purchase plan. Cash provided by financing activities in 2012 primarily reflects proceeds from issuance of debt of \$7.5 million and proceeds from issuance of shares of \$6.4 million, partially offset by repayment of debt of \$10 million. None of the cash used in financing activities for 2012 related to discontinued operations.

Cash provided by financing activities in 2011 primarily reflects \$30.0 million of proceeds from the issuance of debt, partially offset by share repurchases of \$0.1 million. None of the cash used in financing activities for 2011 related to discontinued operations.

### Contingent liabilities

#### Pharmacoepia

In connection with the acquisition of Pharmacoepia in December 2008, Pharmacoepia security holders received a CVR that entitled them to an aggregate cash payment of \$15.0 million under certain circumstances. The CVR expired on December 31, 2011.

#### Neurogen

In connection with the acquisition of Neurogen in December 2009, Neurogen security holders received CVRs under four CVR agreements. The CVRs entitle Neurogen shareholders to cash payments upon the sale or licensing of certain assets and upon the achievement of a specified clinical milestone. The fair value of the Neurogen CVRs at December 31, 2011 was \$0.7 million and related to programs for H3 and VR1. In 2012, we received a notice from a collaborative partner that it was terminating its agreement related to VR1 for convenience and we recorded a decrease in the fair value of the liability for the related CVR of \$0.2 million. Additionally, per the CVR agreement, no payment event date related to the H3 asset can occur after December 23, 2012 and we recorded a decrease in the fair value of the liability for the related CVR of \$0.5 million as of that date. There are no remaining CVR obligations under the agreement with the former Neurogen shareholders.

#### Metabasis

In January 2010, we completed our acquisition of Metabasis. In addition to cash consideration, we issued four tradable CVRs, one CVR from each of four respective series of CVRs, for each Metabasis share. The CVRs will entitle the holder to cash payments as frequently as every six months as cash is received by us from the sale or partnering of any of the Metabasis drug development programs, among other triggering events. Additionally, there were spending requirement obligations related



## Table of Contents

to development funding on the Metabasis programs which have been fulfilled. The fair value of the liability at December 31, 2013, 2012 and 2011 was \$4.2 million, \$0, and \$1.1 million, respectively.

In January 2011, we granted licenses to Chiva to begin immediate development in China of two clinical-stage HepDirect programs, Pradefovir for hepatitis B and MB01733 for hepatocellular carcinoma. Additionally, we granted Chiva a non-exclusive HepDirect technology license for the discovery, development and worldwide commercialization of new compounds in hepatitis B (HepB), hepatitis C (HepC) and hepatocellular carcinoma (HCC). Under the terms of the agreement, we are entitled to milestones and royalties on potential sales. In addition, we are entitled to receive a portion of any sublicensing revenue generated from sublicensing of collaboration compounds to third parties in a major world market. We received a \$0.5 million license payment in March 2011, of which \$0.1 million was remitted to CVR holders. In August 2011, we entered into an amendment to the license agreement which required that a second \$0.5 million licensing fee be paid in September 2011. In addition, the amendment increased royalty rates which we may receive under the license agreement to 6% of net sales of products (other than Pradefovir) and 9% of net sales for Pradefovir. In addition, the amendment removed from the license agreement a provision that afforded us the potential to earn a 10% equity position in Chiva as a milestone payment. In September 2011, Chiva paid us the \$0.5 million licensing fee called for by the amendment, of which \$0.1 million was remitted to CVR holders.

### CyDex

In connection with the acquisition of CyDex on January 24, 2011, we issued a series of CVRs and also assumed certain contingent liabilities. In 2011, \$0.9 million was paid to the CyDex shareholders upon completion of a licensing agreement with MedCo for the Captisol enabled intravenous formulation of Clopidogrel. An additional \$2.0 million was paid to the CyDex shareholders upon acceptance by the FDA of Onyx's NDA, \$4.3 million was paid in January 2012 under the terms of the agreement, and an additional \$3.5 million was paid upon approval by the FDA of Kyprolis for the potential treatment of patients with relapsed and refractory multiple myeloma. We recorded a cash payment of \$0.1 million for the Topiramate orphan drug designation milestone to former license holders. We may be required to make additional payments upon achievement of certain clinical and regulatory milestones to the CyDex shareholders and former license holders. In addition, we will pay CyDex shareholders, for each respective year from 2014 through 2016, (i) 20% of all CyDex-related revenue, but only to the extent that and beginning only when CyDex-related revenue for such year exceed \$15.0 million, (ii) plus an additional 10% of all CyDex-related revenue recognized during such year, but only to the extent that and beginning only when aggregate CyDex-related revenue for such year exceeds \$35.0 million. We paid \$0.2 million to the CyDex shareholders in March 2012 related to 2011 CyDex-related revenue. There was no revenue sharing payment for 2012. The revenue sharing payment for 2013 was \$2.5 million, \$0.9 million of which was paid during the year ended December 31, 2013. The estimated fair value of the contingent liabilities recorded as part of the CyDex acquisition at December 31, 2013, 2012, and 2011 was \$9.3 million, \$10.9 million and \$15.5 million, respectively.

### Leases and Off-Balance Sheet Arrangements

We lease our office and research facilities under operating lease arrangements with varying terms through November 2019. The agreements provide for increases in annual rents based on changes in the Consumer Price Index or fixed percentage increases ranging from 3.0% to 3.5%. We also sublease a portion of our facilities through leases which expire between 2014 and 2016. The sublease agreements provide for a 3% increase in annual rents. We had no off-balance sheet arrangements at December 31, 2013 and 2012.



Table of Contents

## Contractual Obligations

As of December 31, 2013, future minimum payments due under our contractual obligations are as follows (in thousands):

	Payments Due by Period				
	Total	Less than 1 year	2-3 years	4-5 years	More than 5 years
Obligations for uncertain tax positions (1)	\$—	\$—	\$—	\$—	\$—
Co-promote termination obligations (2)	\$ 11,746	\$ 4,329	\$ 6,399	\$ 1,018	\$—
Purchase obligations (3)	\$ 7,215	\$ 7,215	\$—	\$—	\$—
Contingent liabilities (4)	\$ 1,618	\$ 1,618	\$—	\$—	\$—
Note and interest payment obligations	\$ 9,364	\$ 9,364	\$—	\$—	\$—
Operating lease obligations (5)	\$ 14,398	\$ 5,524	\$ 7,045	\$ 1,455	\$ 374

(1) Expected payments related to obligations for uncertain tax positions cannot be reasonably estimated

Co-promote termination obligations represent our legal obligation as primary obligor to Organon due to the fact that Organon did not consent to the legal assignment of the co-promote termination obligation to Pfizer. The liability is offset by an asset which represents a non-interest bearing receivable for future payments to be made by Pfizer.

(3) Purchase obligations represent our commitments under our supply agreement with Hovione for Captisol purchases. Contingent liabilities to former shareholders and licenseholders are subjective and affected by changes in inputs to the valuation model including management's assumptions regarding revenue volatility, probability of commercialization of products, estimates of timing and probability of achievement of certain revenue thresholds and developmental and regulatory milestones and affect amounts owed to former license holders and CVR holders. As of December 31, 2013, only those liabilities for revenue sharing payments achieved as a result of 2013 income are included in the table above.

We lease office and research facilities that we have fully vacated under operating lease arrangements expiring in July 2015 and August 2016. We sublet portions of these facilities through the end of our lease. As of December 31, 2013, we expect to receive aggregate future minimum lease payments totaling \$2.4 million (nondiscounted) over the duration of the sublease agreement as follows and not included in the table above: less than one year, \$1.3 million; two to three years, \$1.1 million; and more than four years, \$0.

## Critical Accounting Policies

Certain of our policies require the application of management judgment in making estimates and assumptions that affect the amounts reported in the consolidated financial statements and disclosures made in the accompanying notes. Those estimates and assumptions are based on historical experience and various other factors deemed to be applicable and reasonable under the circumstances. The use of judgment in determining such estimates and assumptions is by nature, subject to a degree of uncertainty. Accordingly, actual results could differ materially from the estimates made. Our critical accounting policies are as follows:

## Revenue Recognition

Royalties on sales of products commercialized by our partners are recognized in the quarter reported by the respective partner. Generally, we receive royalty reports from our licensees approximately one quarter in arrears due to the fact that our agreements require partners to report product sales between 30-60 days after the end of the quarter. We recognize royalty revenues when we can reliably estimate such amounts and collectability is reasonably assured. Under this accounting policy, the royalty revenues reported are not based upon estimates and such royalty revenues are typically reported in the same period in which payment is received.

Revenue from material sales of Captisol is recognized upon transfer of title, which normally passes upon shipment to the customer. Our credit and exchange policy includes provisions for the return of product between 30 to 90 days, depending on the specific terms of the individual agreement, when that product (1) does not meet specifications, (2) is damaged in shipment (in limited circumstances where title does not transfer until delivery), or (3) is exchanged for an alternative grade of Captisol.

Revenue from research funding under our collaboration agreements is earned and recognized on a percentage-of-completion basis as research hours are incurred in accordance with the provisions of each agreement. Nonrefundable, up-front license fees and milestone payments with standalone value that are not dependent on any future performance by us under our collaboration agreements are recognized as revenue upon the earlier of when payments are received or collection is assured, but are deferred if we have continuing performance obligations. Amounts received under multiple-element arrangements requiring ongoing services or performance by us are recognized over the period of such

## Table of Contents

services or performance. We occasionally have sub-license obligations related to arrangements for which we receive license fees, milestones and royalties. We evaluate the determination of gross versus net reporting based on each individual agreement.

Sales-based milestone revenue is accounted for similarly to royalties, with revenue recognized upon achievement of the milestone assuming all other revenue recognition criteria for milestones are met. Revenue from development and regulatory milestones is recognized when earned, as evidenced by written acknowledgement from the collaborator, provided that (i) the milestone event is substantive, its achievability was not reasonably assured at the inception of the agreement, and we have no further performance obligations relating to that event, and (ii) collectability is reasonably assured. If these criteria are not met, the milestone payment is recognized over the remaining period of our performance obligations under the arrangement.

We analyze our revenue arrangements and other agreements to determine whether there are multiple elements that should be separated and accounted for individually or as a single unit of accounting. For multiple element contracts, arrangement consideration is allocated at the inception of the arrangement to all deliverables on the basis of relative selling price, using a hierarchy to determine selling price. Management first considers vendor-specific objective evidence ("VSOE"), then third-party evidence ("TPE") and if neither VSOE nor TPE exist, we use our best estimate of selling price.

Many of our revenue arrangements involve the bundling of a license with the option to purchase manufactured product. Licenses are granted to pharmaceutical companies for the use of Captisol in the development of pharmaceutical compounds. The licenses may be granted for the use of the Captisol product for all phases of clinical trials and through commercial availability of the host drug or may be limited to certain phases of the clinical trial process. We believe that our licenses have stand-alone value at the outset of an arrangement because the customer obtains the right to use Captisol in its formulations without any additional input by us, and in a hypothetical stand-alone transaction, the customer would be able to procure inventory from another manufacturer in the absence of contractual provisions for exclusive supply by us.

### Inventory

Inventory is stated at the lower of cost or market value. We determine cost using the first-in, first-out method. We analyze our inventory levels periodically and write down inventory to its net realizable value if it has become obsolete, has a cost basis in excess of its expected net realizable value or is in excess of expected requirements.

### Co-Promote Termination Accounting

As part of the termination and return of co-promotion rights agreement that we entered into with Organon in January 2006, we agreed to make quarterly payments to Organon, effective beginning in the fourth quarter of 2006, equal to 6.5% of Avinza net sales through December 31, 2012 and thereafter 6% through patent expiration, currently anticipated to be November 2017. The estimated fair value of the amounts to be paid to Organon after the termination, based on the future estimated net sales of the product, was recognized as a liability and expensed as a cost of the termination as of the effective date of the agreement.

In connection with the Avinza sale transaction, King Pharmaceuticals, now a subsidiary of Pfizer, assumed our obligation to make payments to Organon based on net sales of Avinza (the fair value of which approximated \$11.7 million as of December 31, 2013). As Organon has not consented to the legal assignment of the co-promote termination obligation from us to Pfizer, we remain liable to Organon in the event of Pfizer's default of this obligation. Therefore, we recorded an asset on February 26, 2007 to recognize Pfizer's assumption of the obligation, while continuing to carry the co-promote termination liability in our consolidated financial statements to recognize our legal obligation as primary obligor to Organon. This asset represents a non-interest bearing receivable for future payments to be made by Pfizer and is recorded at its fair value. As of December 31, 2013 and thereafter, the receivable and liability will remain equal and adjusted each quarter for changes in the fair value of the obligation. On a quarterly basis, management reviews the carrying value and assesses the co-promote termination receivable for impairment (e.g. in the event Pfizer defaults on the assumed obligation to pay Organon). Annually management also reviews the carrying value of the co-promote termination liability. Due to assumptions and judgments inherent in determining the estimates of future net Avinza sales through November 2017, the actual amount of net Avinza sales used to determine the amount of the asset and liability for a particular period may be materially different from current estimates. Any

resulting changes to the co-promote termination liability will have a corresponding impact on the co-promote termination payments receivable. As of December 31, 2013 and 2012, the fair value of the co-promote termination liability (and the corresponding receivable) was determined using a discount rate of 15%.

Table of Contents

## Impairment of Long-Lived Assets

We review long-lived assets for impairment annually or whenever events or circumstances indicate that the carrying amount of the assets may not be recoverable. We measure the recoverability of assets to be held and used by comparing the carrying amount of an asset to future undiscounted net cash flows expected to be generated by the asset. If such assets are considered to be impaired, the impairment to be recognized is measured as the amount by which the carrying amount of the assets exceeds the fair value of the assets. Fair value of our long-lived assets is determined using the expected cash flows discounted at a rate commensurate with the risk involved. As of December 31, 2013, we believe that the future discounted cash flows to be received from our long-lived assets will exceed the assets' carrying value.

## Income Taxes

Income taxes are accounted for under the asset and liability method. This approach requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of differences between the tax basis of assets or liabilities and their carrying amounts in the consolidated financial statements. A valuation allowance is provided for deferred tax assets if it is more likely than not that these items will either expire before we are able to realize their benefit or if future deductibility is uncertain. As of December 31, 2013, we have provided a full valuation allowance against our deferred tax assets as recoverability was uncertain. Developing the provision for income taxes requires significant judgment and expertise in federal and state income tax laws, regulations and strategies, including the determination of deferred tax assets and liabilities and, if necessary, any valuation allowances that may be required for deferred tax assets. Our judgments and tax strategies are subject to audit by various taxing authorities. While we believe we have provided adequately for our income tax liabilities in our consolidated financial statements, adverse determinations by these taxing authorities could have a material adverse effect on our consolidated financial condition and results of operations. Our ending deferred tax liability represents liabilities for which we cannot estimate the reversal period and therefore cannot be used as support for our deferred tax assets.

## Share-Based Compensation

Share-based compensation cost for awards to employees and non-employee directors is recognized on a straight-line basis over the vesting period until the last tranche vests. Compensation cost for consultant awards is recognized over each separate tranche's vesting period. We recognized compensation expense of \$5.7 million, \$4.1 million and \$3.4 million for 2013, 2012 and 2011, respectively, associated with option awards, restricted stock and our employee stock purchase plan.

The fair-value for options that were awarded to employees and directors was estimated at the date of grant using the Black-Scholes option valuation model with the following weighted average assumptions:

	Year Ended December 31,		
	2013	2012	2011
Risk-free interest rate	1.13%-1.82%	0.83%-1.14%	1.09%-2.61%
Dividend yield	—	—	—
Expected volatility	70%	69%	69%
Expected term	6 years	6 years	6 years
Forfeiture rate	8.4%-9.8%	8.0%-11.2%	8.9%-14.1%

The risk-free interest rate is based on the U.S. Treasury yield curve at the time of the grant. The expected term of the employee and non-employee director options is the estimated weighted-average period until exercise or cancellation of vested options (forfeited unvested options are not considered) based on historical experience. The expected term for consultant awards is the remaining period to contractual expiration. Volatility is a measure of the expected amount of variability in the stock price over the expected life of an option expressed as a standard deviation. In making this assumption, we used the historical volatility of our stock price over a period equal to the expected term. The forfeiture rate is based on historical data at the time of the grant.

## New Accounting Pronouncements

In July 2012, the Financial Accounting Standards Board, or FASB, issued Accounting Standards Update, or ASU, 2012-02, Intangibles - Goodwill and Other: Testing Indefinite-Lived Intangible Assets for Impairment in ASU

2012-02. ASU 2012-02 allows a company the option to first assess qualitative factors to determine whether it is necessary to perform a quantitative impairment test. Under that option, a company would no longer be required to calculate the fair value of an indefinite-lived intangible asset unless the company determines, based on that qualitative assessment, that it is more likely than not that the fair value of the indefinite-lived intangible asset is less than its carrying amount. The amendments in this ASU are

## Table of Contents

effective for annual and interim indefinite-lived intangible asset impairment tests performed for periods beginning after September 15, 2012. We adopted this standard for the year ended December 31, 2012. The adoption of ASU 2012-02 did not have a material impact on our financial position or results of operations.

In February 2013, the FASB issued ASU No. 2013-02, Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income. Under ASU 2013-02, an entity is required to provide information about the amounts reclassified out of Accumulated Other Comprehensive Income, or AOCI, by component. In addition, an entity is required to present, either on the face of the financial statements or in the notes, significant amounts reclassified out of AOCI by the respective line items of net income, but only if the amount reclassified is required to be reclassified in its entirety in the same reporting period. For amounts that are not required to be reclassified in their entirety to net income, an entity is required to cross-reference to other disclosures that provide additional details about those amounts. Implementing ASU 2013-02 did not change the current requirements for reporting net income or other comprehensive income in the financial statements. The amendments in this ASU are effective for us for fiscal years, and interim periods within those years, beginning after January 1, 2014.

In July, 2013, the FASB issued Accounting Standards Update No. 2013-11, Presentation of an Unrecognized Tax Benefit When a Net Operating Loss Carryforward, a Similar Tax Loss, or a Tax Credit Carryforward Exists. ASU 2013-11 requires the netting of unrecognized tax benefits, or UTBs, against a deferred tax asset for a loss or other carryforward that would apply in settlement of the uncertain tax positions. UTBs are required to be netted against all available same-jurisdiction loss or other tax carryforwards that would be utilized, rather than only against carryforwards that are created by the UTBs. ASU 2013-11 is effective for us for interim and annual periods beginning after December 15, 2013. We are currently evaluating the effect, if any, the adoption of this standard will have on our financial statements.

### Item 7A. Quantitative and Qualitative Disclosures About Market Risk

At December 31, 2013, our investment portfolio included investments in available for sale equity securities of \$4.3 million. These securities are subject to market risk and may decline in value based on market conditions.

We do not hold or issue derivatives, derivative commodity instruments or other financial instruments for speculative trading purposes. Further, we do not believe our cash and cash equivalents and restricted cash and investments have significant risk of default or illiquidity. We made this determination based on discussions with our investment advisors and a review of our holdings. While we believe our cash and cash equivalents and restricted cash and investments do not contain excessive risk, we cannot provide absolute assurance that in the future our investments will not be subject to adverse changes in market value. All of our cash and cash equivalents and restricted cash and investments are held at fair value.

We purchase Captisol from Hovione, located in Lisbon, Portugal. Payments to Hovione are denominated and paid in U.S. dollars, however the unit price of Captisol contains an adjustment factor which is based on the sharing of foreign currency risk between the two parties. The effect of an immediate 10% change in foreign exchange rates would have an immaterial impact on our financial condition, results of operations or cash flows.

We are exposed to market risk involving rising interest rates. To the extent interest rates rise, our interest costs could increase. An increase in interest costs of 10% would have no material impact on our financial condition, results of operations or cash flows.

### Item 8. Consolidated Financial Statements and Supplementary Data

Table of Contents

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

	Page
<u>Report of Independent Registered Public Accounting Firm</u>	<u>41</u>
<u>Consolidated Balance Sheets</u>	<u>42</u>
<u>Consolidated Statements of Operations</u>	<u>43</u>
<u>Consolidated Statements of Comprehensive Income (Loss)</u>	<u>44</u>
<u>Consolidated Statements of Stockholders' Equity (Deficit)</u>	<u>45</u>
<u>Consolidated Statements of Cash Flows</u>	<u>46</u>
<u>Notes to Consolidated Financial Statements</u>	<u>48</u>

40

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Table of Contents

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

Board of Directors and Shareholders

Ligand Pharmaceuticals Incorporated

We have audited the accompanying consolidated balance sheets of Ligand Pharmaceuticals Incorporated (the “Company”) as of December 31, 2013 and 2012, and the related consolidated statements of operations, comprehensive income (loss), stockholders’ equity (deficit), and cash flows for each of the three years in the period ended December 31, 2013. These financial statements are the responsibility of the Company’s management. Our responsibility is to express an opinion on these 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 financial position of Ligand Pharmaceuticals Incorporated as of December 31, 2013 and 2012, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2013 in conformity with accounting principles generally accepted in the United States of America.

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 February 24, 2014 expressed an unqualified opinion.

/s/ GRANT THORNTON LLP

Los Angeles, California

February 24, 2014

Table of Contents

LIGAND PHARMACEUTICALS INCORPORATED  
CONSOLIDATED BALANCE SHEETS  
(in thousands, except share data)

	December 31, 2013	2012
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 11,639	\$ 12,381
Short-term investments	4,340	—
Accounts receivable, net	2,222	4,589
Inventory	1,392	1,697
Other current assets	959	829
Current portion of co-promote termination payments receivable	4,329	4,327
Total current assets	24,881	23,823
Restricted cash and investments	1,341	2,767
Property and equipment, net	867	788
Deferred income taxes	—	8
Intangible assets, net	53,099	55,912
Goodwill	12,238	12,238
Commercial license rights	4,571	—
Long-term portion of co-promote termination payments receivable	7,417	8,207
Other assets	299	517
Total assets	\$ 104,713	\$ 104,260
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 3,951	\$ 5,854
Accrued liabilities	5,337	4,961
Current portion of contingent liabilities	1,712	356
Current portion of deferred income taxes	1,574	1,581
Current portion of note payable	9,109	14,835
Current portion of co-promote termination liability	4,329	4,327
Current portion of lease exit obligations	2,811	3,039
Current portion of deferred revenue	116	486
Total current liabilities	28,939	35,439
Long-term portion of note payable	—	13,443
Long-term portion of co-promote termination liability	7,417	8,207
Long-term portion of deferred revenue, net	2,085	2,369
Long-term portion of lease exit obligations	3,071	5,963
Long-term portion of deferred income taxes	1,098	725
Long-term portion of contingent liabilities	11,795	10,543
Other long-term liabilities	695	1,086
Total liabilities	55,100	77,775
Commitments and contingencies-see note		
Stockholders' equity:		
Common stock, \$0.001 par value; 33,333,333 shares authorized; 20,468,521 and 21,278,606 shares issued and outstanding at December 31, 2013 and 2012, respectively	21	21
Additional paid-in capital	718,017	751,503

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Accumulated other comprehensive income	2,914	—	
Accumulated deficit	(671,339	) (682,759	)
Treasury stock, at cost; 0 and 1,118,222 shares at December 31, 2013 and 2012, respectively	—	(42,280	)
Total stockholders' equity	49,613	26,485	
Total liabilities and stockholders' equity	\$ 104,713	\$ 104,260	
See accompanying notes to these consolidated financial statements.			

Table of Contents

LIGAND PHARMACEUTICALS INCORPORATED  
CONSOLIDATED STATEMENTS OF OPERATIONS  
(in thousands, except share data)

	Year Ended December 31,		
	2013	2012	2011
Revenues:			
Royalties	\$23,584	\$14,073	\$9,213
Material Sales	19,072	9,432	12,123
Collaborative research and development and other revenues	6,317	7,883	8,701
Total revenues	48,973	31,388	30,037
Operating costs and expenses:			
Cost of material sales	5,732	3,601	4,909
Research and development	9,274	10,790	10,291
General and administrative	17,984	15,782	14,583
Lease exit and termination costs	560	1,022	552
Write-off of acquired in-process research and development	480	—	2,282
Total operating costs and expenses	34,030	31,195	32,617
Accretion of deferred gain on sale leaseback	—	—	1,702
Income (loss) from operations	14,943	193	(878 )
Other (expense) income:			
Interest expense, net	(2,077 )	(2,924 )	(2,297 )
Increase in contingent liabilities	(3,597 )	(1,650 )	(1,013 )
Other, net	(63 )	516	630
Total other expense, net	(5,737 )	(4,058 )	(2,680 )
Income (loss) from continuing operations before income tax benefit	9,206	(3,865 )	(3,558 )
Income tax (expense) benefit from continuing operations	(374 )	1,191	13,270
Income (loss) from continuing operations	8,832	(2,674 )	9,712
Discontinued operations:			
Gain on sale of Avinza Product Line, net	2,588	3,656	—
Gain on sale of Oncology Product Line, net	—	—	3
Income tax expense on discontinued operations	—	(1,509 )	—
Income from discontinued operations	2,588	2,147	3
Net income (loss)	\$11,420	\$(527 )	\$9,715
Basic per share amounts:			
Income (loss) from continuing operations	\$0.43		