Aeterna Zentaris Inc.
Form 20-F
March 21, 2014

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

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FORM 20-F

¨Registration Statement Pursuant to Section 12(b) or 12(g) of The Securities Exchange Act of 1934

OR

ýAnnual 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

OR

¨Shell Company Report Pursuant to Section 13 or 15(d) of The Securities Exchange Act of 1934

Commission file number 0-30752

AETERNA ZENTARIS INC.
(Exact Name of Registrant as Specified in its Charter)

Not Applicable
(Translation of Registrant's Name into English)

Canada
(Jurisdiction of Incorporation)

1405 du Parc-Technologique Blvd.
Quebec City, Quebec
Canada, G1P 4P5
(Address of Principal Executive Offices)

Dennis Turpin
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E-mail: dturpin@aezsinc.com
1405 du Parc-Technologique Blvd.
Quebec City, Quebec
Canada, G1P 4P5
(Name, Telephone, E-mail and Address of Company Contact Person)

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

Title of Each Class	Name of Each Exchange on Which Registered
Common Shares	NASDAQ Capital Market Toronto Stock Exchange

Securities registered or to be registered pursuant to Section 12(g) of the Act: NONE

Securities for which there is a reporting obligation pursuant to Section 15(d) of the ACT: NONE

Indicate the number of outstanding shares of each of the issuer's classes of capital or common stock as at the close of the period covered by the annual report: 45,312,009 Common Shares as at December 31, 2013.

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

If this report is an annual or transition report, indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934. Yes ¨ No ý

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 ý No ¨

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

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

Large accelerated filer ¨ Accelerated filer ý Non-accelerated filer ¨

Indicate by check mark which basis of accounting the registrant has used to prepare the financial statements included in this filing:

US GAAP ¨ International Financial Reporting Standards as issued by the Other ¨
International Accounting Standards Board ý

If "other" has been checked in response to the previous question, indicate by check mark which financial statement item the registrant has elected to follow. Item 17 ¨ Item 18 ¨

If this is an annual report, indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ¨ No ý

Basis of Presentation

General

Except where the context otherwise requires, all references in this annual report on Form 20-F to the "Company", "Aeterna Zentaris Inc.", "we", "us", "our" or similar words or phrases are to Aeterna Zentaris Inc. and its subsidiaries, taken together. In this annual report on Form 20-F, references to "$" and "US$" are to United States dollars, references to "CAN$" are to Canadian dollars and references to "EUR" are to euros. Unless otherwise indicated, the statistical and financial data contained in this annual report on Form 20-F are presented as at December 31, 2013.

This annual report on Form 20-F also contains certain information regarding products or product candidates that may potentially compete with our products and product candidates, and such information has been primarily derived from information made publicly available by the companies developing such potentially competing products and product candidates and has not been independently verified by Aeterna Zentaris Inc.

Forward-Looking Statements

This annual report on Form 20-F contains forward-looking statements made pursuant to the safe harbor provisions of the U.S. Securities Litigation Reform Act of 1995. Forward-looking statements can be identified by words such as: "intend," "believe," "designed to," "vision," "aimed at," "expect," "may," "should," "would," "will" and similar references. Such statements include, but are not limited to, statements about the progress of our research, development and clinical trials and the timing of, and prospects for, regulatory approval and commercialization of our product candidates, the timing of expected results of our studies and anticipated results of these studies, and estimates regarding our capital requirements and our needs for, and our ability to obtain, additional financing. Forward-looking statements involve known and unknown risks and uncertainties, which could cause the Company's actual results to differ materially from those in the forward-looking statements. Such risks and uncertainties include, among others, the availability of funds and resources to pursue our research and development ("R&D") projects, the successful and timely completion of clinical studies, the degree of market acceptance once our products are approved for commercialization, the ability of the Company to take advantage of business opportunities in the pharmaceutical industry, the ability of the Company to protect its intellectual property, uncertainties related to the regulatory process and general changes in economic conditions. Investors should consult the Company's quarterly and annual filings with the Canadian and United States ("U.S.") securities commissions for additional information on risks and uncertainties relating to the forward-looking statements. Investors are cautioned not to rely on these forward-looking statements. The Company does not undertake to update these forward-looking statements and disclaims any obligation to update any such factors or to publicly announce the result of any revisions to any of the forward-looking statements contained herein to reflect future results, events or developments except if required to do so by a governmental authority or applicable law.

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PART I

Item 1. Identity of Directors, Senior Management and Advisers

A. Directors and senior management

Not applicable.

B. Advisers

Not applicable.

C. Auditors

Not applicable.

Item 2. Offer Statistics and Expected Timetable

A. Offer statistics

Not applicable.

B. Method and expected timetable

Not applicable.

Item 3. Key Information

A. Selected financial data

The consolidated statement of comprehensive income (loss) data set forth in this Item 3.A with respect to the years ended December 31, 2013, 2012 and 2011 and the consolidated statement of financial position data as at December 31, 2013 and 2012 have been derived from the audited consolidated financial statements listed in Item 18, which have been prepared in accordance with International Financial Reporting Standards ("IFRS"), as issued by the International Accounting Standards Board ("IASB"). The consolidated statement of financial position data as at December 31, 2011 set forth in this Item 3.A have been derived from our previous consolidated financial statements not included herein, and have also been prepared in accordance with IFRS, as issued by the IASB. The selected financial data should be read in conjunction with our audited consolidated financial statements and the related notes included elsewhere in this annual report on Form 20-F, as well as "Item 5. – Operating and Financial Review and Prospects" of this annual report on Form 20-F.

Consolidated Statements of Comprehensive Income (Loss)
(in thousands of US dollars, except share and per share data)
Derived from consolidated financial statements prepared in accordance with IFRS

	Years ended December 31,		
	2013	2012	2011
	$	$	$
Revenues			
Sales	96	834	250
License fees and other	6,079	1,219	4,455
	6,175	2,053	4,705
Operating expenses			
Cost of sales	51	591	212
Research and development costs, net of refundable tax credits and grants	21,284	20,592	24,245
Selling, general and administrative expenses	12,316	10,606	11,955
	33,651	31,789	36,412
Loss from operations	(27,476)	(29,736)	(31,707)
Finance income	1,748	6,974	6,239
Finance costs	(1,512)	(382)	(8)
Net finance income	236	6,592	6,231
Loss before income taxes	(27,240)	(23,144)	(25,476)
Income tax expense	—	—	(1,104)
Net loss from continuing operations	(27,240)	(23,144)	(26,580)
Net income (loss) from discontinued operations	34,055	2,732	(487)
Net income (loss)	6,815	(20,412)	(27,067)
Other comprehensive income (loss):			
Items that may be reclassified subsequently to profit or loss:			
Foreign currency translation adjustments	1,073	(504)	(789)
Items that will not be reclassified to profit or loss:			
Actuarial gain (loss) on defined benefit plans	2,346	(3,705)	(1,335)
Comprehensive income (loss)	10,234	(24,621)	(29,191)
Net loss per share (basic and diluted) from continuing operations	(0.92)	(1.17)	(1.69)
Net income (loss) (basic and diluted) from discontinued operations	1.16	0.14	(0.03)
Net income (loss) (basic and diluted) per share	0.24	(1.03)	(1.72)
Weighted average number of shares outstanding:			
Basic	29,476,455	19,775,073	15,751,331
Diluted	29,476,455	19,806,687	15,751,331

Consolidated Statement of Financial Position Information
(in thousands of US dollars)
Derived from consolidated financial statements prepared in accordance with IFRS

	As at December 31,		
	2013	2012	2011
	$	$	$
Cash and cash equivalents	43,202	39,521	46,881
Restricted cash equivalents	865	826	806
Total assets	59,196	67,655	75,369
Warrant liability (current and non-current)	18,010	6,176	9,204
Share capital	134,101	122,791	101,884
Shareholders' equity (deficiency)	17,064	(6,695)	(4,546)

B. Capitalization and indebtedness

Not applicable.

C. Reasons for the offer and use of proceeds

Not applicable.

D. Risk factors

Risks Relating to Us and Our Business

Investments in biopharmaceutical companies are generally considered to be speculative.

The prospects for companies operating in the biopharmaceutical industry may generally be considered to be uncertain, given the very nature of the industry and, accordingly, investments in biopharmaceutical companies should be considered to be speculative.

We have a history of operating losses and we may never achieve or maintain operating profitability.

Our product candidates remain at the development stage, and we have incurred substantial expenses in our efforts to develop products. Consequently, we have incurred recurrent operating losses and, as disclosed in our audited consolidated financial statements as at December 31, 2013 and December 31, 2012 and for the years ended December 31, 2013, 2012 and 2011, we had an accumulated deficit of $203.9 million as at December 31, 2013. Our operating losses have adversely impacted, and will continue to adversely impact, our working capital, total assets and shareholders' equity (deficiency). We do not expect to reach operating profitability in the immediate future, and our operating expenses are likely to continue to represent a significant component of our overall cost profile as we continue our R&D and clinical study programs and seek regulatory approval for our product candidates. Even if we succeed in developing, acquiring or in-licensing new commercial products, we could incur additional operating losses for at least the next several years. If we do not ultimately generate sufficient revenue from commercialized products and achieve or maintain operating profitability, an investment in our Common Shares could result in a significant or total loss.

Our clinical trials may not yield results which will enable us to obtain regulatory approval for our products, and a setback in any of our clinical trials would likely cause a drop in the price of our Common Shares.

We will only receive regulatory approval for a product candidate if we can demonstrate in carefully designed and conducted clinical trials that the product candidate is both safe and effective. We do not know whether our pending or any future clinical trials will demonstrate sufficient safety and efficacy to obtain the requisite regulatory approvals or will result in marketable products. Unfavorable data from those studies could result in the withdrawal of marketing approval for approved products or an extension of the review period for developmental products. Clinical trials are inherently lengthy, complex, expensive and uncertain processes and have a high risk of failure. It typically takes many years to complete testing, and failure can occur at any stage of testing. Results attained in preclinical testing and early clinical studies, or trials, may not be indicative of results that are obtained in later studies.

None of our current product candidates has to date received regulatory approval for its intended commercial sale. We cannot market a pharmaceutical product in any jurisdiction until it has completed rigorous preclinical testing and clinical trials and passed such jurisdiction's extensive regulatory approval process. In general, significant R&D and clinical studies are required to demonstrate the safety and efficacy of our product candidates before we can submit regulatory applications. Preclinical testing and clinical development are long, expensive and uncertain processes. Preparing, submitting and advancing applications for regulatory approval is complex, expensive and time-consuming and entails significant uncertainty. Data obtained from preclinical and clinical tests can be interpreted in different ways, which could delay, limit or prevent regulatory approval. It may take us many years to complete the testing of our product candidates and failure can occur at any stage of this process. In addition, we have limited experience in conducting and managing the clinical trials necessary to obtain regulatory approval in the U.S., in Canada and abroad and, accordingly, may encounter unforeseen problems and delays in the approval process. Though we may engage a contract research organization (a "CRO") with experience in conducting regulatory trials, errors in the conduct, monitoring and/or auditing could invalidate the results from a regulatory perspective. Even if a product candidate is approved by the United States Food and Drug Administration (the "FDA"), the Canadian Therapeutic Products Directorate or any other regulatory authority, we may not obtain approval for an indication whose market is large enough to recover our investment in that product candidate. In addition, there can be no assurance that we will ever obtain all or any required regulatory approvals for any of our product candidates.

We are currently developing our product candidates based on R&D activities, preclinical testing and clinical trials conducted to date, and we may not be successful in developing or introducing to the market these or any other new products or technology. If we fail to develop and deploy new products successfully and on a timely basis, we may become non-competitive and unable to recover the R&D and other expenses we incur to develop and test new products.

Interim results of preclinical or clinical studies do not necessarily predict their final results, and acceptable results in early studies might not be obtained in later studies. Safety signals detected during clinical studies and preclinical animal studies may require us to perform additional studies, which could delay the development of the drug or lead to a decision to discontinue development of the drug. Product candidates in the later stages of clinical development may fail to show the desired safety and efficacy traits despite positive results in initial clinical testing. Results from earlier studies may not be indicative of results from future clinical trials and the risk remains that a pivotal program may generate efficacy data that will be insufficient for the approval of the drug, or may raise safety concerns that may prevent approval of the drug. Interpretation of the prior preclinical and clinical safety and efficacy data of our product candidates may be flawed and there can be no assurance that safety and/or efficacy concerns from the prior data were overlooked or misinterpreted, which in subsequent, larger studies appear and prevent approval of such product candidates.

Furthermore, we may suffer significant setbacks in advanced clinical trials, even after promising results in earlier studies. Based on results at any stage of clinical trials, we may decide to repeat or redesign a trial or discontinue development of one or more of our product candidates. Further, actual results may vary once the final and quality-controlled verification of data and analyses has been completed. If we fail to adequately demonstrate the safety and efficacy of our products under development, we will not be able to obtain the required regulatory approvals to commercialize our product candidates.

Clinical trials are subject to continuing oversight by governmental regulatory authorities and institutional review boards and must:

- meet the requirements of these authorities;
- meet the requirements for informed consent; and
- meet the requirements for good clinical practices.

We may not be able to comply with these requirements in respect of one or more of our product candidates.

In addition, we rely on third parties, including CROs and outside consultants, to assist us in managing and monitoring clinical trials. Our reliance on these third parties may result in delays in completing, or in failing to complete, these trials if one or more third parties fails to perform with the speed and level of competence we expect.

A failure in the development of any one of our programs or product candidates could have a negative impact on the development of the others. Setbacks in any phase of the clinical development of our product candidates would have an adverse financial impact (including with respect to any agreements and partnerships that may exist between us and other entities), could jeopardize regulatory approval and would likely cause a drop in the price of our securities.

If we are unable to successfully complete our clinical trial programs, or if such clinical trials take longer to complete than we project, our ability to execute our current business strategy will be adversely affected.

Whether or not and how quickly we complete clinical trials is dependent in part upon the rate at which we are able to engage clinical trial sites and, thereafter, the rate of enrollment of patients, and the rate we collect, clean, lock and analyze the clinical trial database. Patient enrollment is a function of many factors, including the design of the protocol, the size of the patient population, the proximity of patients to and availability of clinical sites, the eligibility criteria for the study, the perceived risks and benefits of the drug under study and of the control drug, if any, the efforts to facilitate timely enrollment in clinical trials, the patient referral practices of physicians, the existence of competitive clinical trials, and whether existing or new drugs are approved for the indication we are studying. Certain clinical trials are designed to continue until a pre-determined number of events have occurred to the patients enrolled. Such trials are subject to delays stemming from patient withdrawal and from lower than expected event rates and may also incur increased costs if enrollment is increased in order to achieve the desired number of events. If we experience delays in identifying and contracting with sites and/or in patient enrollment in our clinical trial programs, we may incur additional costs and delays in our development programs, and may not be able to complete our clinical trials on a cost-effective or timely basis. In addition, conducting multi-national studies adds another level of complexity and risk as we are subject to events affecting countries outside Canada. Moreover, negative or inconclusive results from the clinical trials we conduct or adverse medical events could cause us to have to repeat or terminate the clinical trials. Accordingly, we may not be able to complete the clinical trials within an acceptable time frame, if at all. If we or any third party have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay or terminate ongoing clinical trials.

Additionally, we have limited experience in filing a New Drug Application ("NDA") or similar application for approval in the U.S. or in any country for our current product candidates, which may result in a delay in, or the rejection of, our filing of an NDA or similar application. During the drug development process, regulatory agencies will typically ask questions of drug sponsors. While we endeavor to answer all such questions in a timely fashion, or in the NDA filing, some questions may not be answered by the time we file our NDA. Unless the FDA waives the requirement to answer any such unanswered questions, submission of an NDA may be delayed and acceptance of an NDA may ultimately be rejected.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be successful in commercializing MACRILEN™ or any other product candidate if and when they are approved.

We currently have a lean sales and marketing staff and have limited recent experience in the sale or marketing of pharmaceutical or biopharmaceutical products. To achieve commercial success for any approved product, including, in the near and medium term, MACRILEN™, we must either develop a sales and marketing organization or outsource these functions to third parties. We currently plan to establish our own sales and marketing capabilities and promote MACRILEN™ with a targeted sales force if and when it is ultimately approved. There are risks involved with establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel and representatives;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales and marketing services, our product revenues or the profitability of these product revenues to us are likely to be lower than if we were to market and sell any products that

we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates and our business, financial condition and results of operations will be materially adversely affected.

We may not be able to successfully integrate acquired businesses or in-licensed products.

Future acquisitions or in-licensed products may not be successfully integrated. The failure to successfully integrate the personnel and operations of businesses that we may acquire or of products that we may in-license in the future with our operations, business and products could have a material adverse effect on our operations and results.

We are and will be subject to stringent ongoing government regulation for our products and our product candidates, even if we obtain regulatory approvals for the latter.

The manufacture, marketing and sale of our products and product candidates are and will be subject to strict and ongoing regulation, even if regulatory authorities approve any of the latter. Compliance with such regulation will be expensive and consume substantial financial and management resources. For example, an approval for a product may be conditioned on our agreement to conduct costly post-marketing follow-up studies to monitor the safety or efficacy of the products. In addition, as a clinical experience with a drug expands after approval because the drug is used by a greater number and more diverse group of patients than during clinical trials, side effects or other problems may be observed after approval that were not observed or anticipated during pre-approval clinical trials. In such a case, a regulatory authority could restrict the indications for which the product may be sold or revoke the product's regulatory approval.

We and our contract manufacturers will be required to comply with applicable current Good Manufacturing Practice ("cGMP") regulations for the manufacture of our products. These regulations include requirements relating to quality assurance, as well as the corresponding maintenance of rigorous records and documentation. Manufacturing facilities must be approved before we can use them in the commercial manufacturing of our products and are subject to subsequent periodic inspection by regulatory authorities. In addition, material changes in the methods of manufacturing or changes in the suppliers of raw materials are subject to further regulatory review and approval.

If we, or if any future marketing collaborators or contract manufacturers, fail to comply with applicable regulatory requirements, we may be subject to sanctions including fines, product recalls or seizures and related publicity requirements, injunctions, total or partial suspension of production, civil penalties, suspension or withdrawals of previously granted regulatory approvals, warning or untitled letters, refusal to approve pending applications for marketing approval of new products or of supplements to approved applications, import or export bans or restrictions, and criminal prosecution and penalties. Any of these penalties could delay or prevent the promotion, marketing or sale of our products and product candidates.

If our products do not gain market acceptance, we may be unable to generate significant revenues.

Even if our products are approved for commercialization, they may not be successful in the marketplace. Market acceptance of any of our products will depend on a number of factors including, but not limited to:

- demonstration of clinical efficacy and safety;
- the prevalence and severity of any adverse side effects;
- limitations or warnings contained in the product's approved labeling;
- availability of alternative treatments for the indications we target;
- the advantages and disadvantages of our products relative to current or alternative treatments;
- the availability of acceptable pricing and adequate third-party reimbursement; and
- the effectiveness of marketing and distribution methods for the products.

If our products do not gain market acceptance among physicians, patients, healthcare payers and others in the medical community, who may not accept or utilize our products, our ability to generate significant revenues from our products would be limited and our financial condition could be materially adversely affected. In addition, if we fail to further penetrate our core markets and existing geographic markets or successfully expand our business into new markets, the growth in sales of our products, along with our operating results, could be negatively impacted.

Our ability to further penetrate our core markets and existing geographic markets in which we compete or to successfully expand our business into additional countries in Europe, Asia or elsewhere is subject to numerous factors, many of which are beyond our control. Our products, if successfully developed, may compete with a number of drugs, therapies, products and tests currently manufactured and marketed by major pharmaceutical and other biotechnology companies. Our products may also compete with new products currently under development by others or with products which may be less expensive than our products. There can be no assurance that our efforts to increase market

penetration in our core markets and existing geographic markets will be successful. Our failure to do so could have an adverse effect on our operating results and would likely cause a drop in the price of our securities.

We may require significant additional financing, and we may not have access to sufficient capital.

We may require additional capital to pursue planned clinical trials, regulatory approvals, as well as further R&D and marketing efforts for our product candidates and potential products. Except as expressly described in this annual report on Form 20-F, we do not anticipate generating significant revenues from operations in the near future and we currently have no committed sources of capital.

We may attempt to raise additional funds through public or private financings, collaborations with other pharmaceutical companies or from other sources, including, without limitation, through at-the-market offerings and issuances of Common Shares. Additional funding may not be available on terms which are acceptable to us. If adequate funding is not available to us on reasonable terms, we may need to delay, reduce or eliminate one or more of our product development programs or obtain funds on terms less favorable than we would otherwise accept. To the extent that additional capital is raised through the sale of equity securities or securities convertible into or exchangeable for equity securities (collectively, "Convertible Securities"), the issuance of those securities could result in dilution to our shareholders. Moreover, the incurrence of debt financing could result in a substantial portion of our future operating cash flow, if any, being dedicated to the payment of principal and interest on such indebtedness and could impose restrictions on our operations. This could render us more vulnerable to competitive pressures and economic downturns.

We anticipate that our existing working capital, including the proceeds from any sale of Common Shares or other securities and anticipated revenues, will be sufficient to fund our development programs, clinical trials and other operating expenses for the near future. However, our future capital requirements are substantial and may increase beyond our current expectations depending on many factors including:

- the duration and results of our clinical trials for our various product candidates going forward;
- unexpected delays or developments in seeking regulatory approvals;
- the time and cost involved in preparing, filing, prosecuting, maintaining and enforcing patent claims;
- other unexpected developments encountered in implementing our business development and commercialization strategies;
- the potential addition of commercialized products to our pipeline;
- the outcome of litigation, if any; and
- further arrangements, if any, with collaborators.

In addition, global economic and market conditions as well as future developments in the credit and capital markets may make it even more difficult for us to raise additional financing in the future.

If we are unsuccessful in increasing our revenues and/or raising additional funding, we may possibly cease to continue operating as we currently do.

We have had sustained losses, accumulated deficits and negative cash flows from operations since our inception and we expect that this will continue for the foreseeable future.

Although our audited consolidated financial statements as at December 31, 2013 and December 31, 2012 and for the years ended December 31, 2013, 2012 and 2011 have been prepared on a going concern basis, which contemplates the realization of assets and liquidation of liabilities during the normal course of operations, our ability to continue as a going concern is dependent on the successful execution of our business plan, which will require an increase in revenue and/or additional funding to be provided by potential investors as well as non-traditional sources of financing.

Although we stated in our audited consolidated financial statements as at December 31, 2013 and December 31, 2012 and for the years ended December 31, 2013, 2012 and 2011 that management believed that the Company had, as at December 31, 2013, sufficient financial resources to fund planned expenditures and other working capital needs for at least, but not limited to, the 12-month period following such date, there can be no assurance that management will be able to reiterate such belief in the future, particularly in the event that we do not or are unable to raise additional capital, as we do not expect our operations to generate sufficient cash flow to fund our obligations.

Additional funding may be in the form of debt or equity or a hybrid instrument depending on our needs, those of investors and market conditions. Depending on the prevailing global economic and credit market conditions, we may not be able to raise additional cash resources through these traditional sources of financing. Although we may also pursue non-traditional sources of financing with third parties, the global credit markets may adversely affect the

ability of potential third parties to pursue such transactions with us. Accordingly, as a result of the foregoing, we continue to review traditional sources of financing, such as private and public debt or various equity financing alternatives, as well as other alternatives to enhance shareholder value including, but not limited to, non-traditional sources of financing, such as alliances with strategic partners, the sale of assets or licensing of our technology or intellectual property, a combination of operating and related initiatives or a substantial reorganization of our business.

There can be no assurance that we will achieve profitability or positive cash flows or be able to obtain additional funding or that, if obtained, they will be sufficient, or whether any other initiatives will be successful, such that we may continue as a going concern. There could also be material uncertainties related to certain adverse conditions and events that could impact our ability to remain a going concern.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications for which there may be a greater likelihood of success.

Because we have limited financial and managerial resources, we are currently focusing our efforts on our later-stage clinical research programs, zoptarelin doxorubicin and macimorelin, and we are doing so for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications for which there may be a greater likelihood of success or may prove to have greater commercial potential. Notwithstanding our investment to date and anticipated future expenditures on zoptarelin doxorubicin, macimorelin and our earlier-stage programs, we have not yet developed, and may never successfully develop, any marketed treatments using these products. Research programs to identify new product candidates or pursue alternative indications for current product candidates require substantial technical, financial and human resources. These activities may initially show promise in identifying potential product candidates or indications, yet fail to yield product candidates or indications for further clinical development.

We may not achieve our projected development goals in the time-frames we announce and expect.

We set goals and make public statements regarding the timing of the accomplishment of objectives material to our success, such as the commencement, enrollment and anticipated completion of clinical trials, anticipated regulatory submission and approval dates and time of product launch. The actual timing of these events can vary dramatically due to factors such as delays or failures in our clinical trials, the uncertainties inherent in the regulatory approval process and delays in achieving manufacturing or marketing arrangements sufficient to commercialize our products. There can be no assurance that our clinical trials will be completed, that we will make regulatory submissions or receive regulatory approvals as planned or that we will be able to adhere to our current schedule for the launch of any of our products. If we fail to achieve one or more of these milestones as planned, the price of our securities would likely decline.

If we fail to obtain acceptable prices or adequate reimbursement for our products, our ability to generate revenues will be diminished.

The ability for us and/or our partners to successfully commercialize our products will depend significantly on our ability to obtain acceptable prices and the availability of reimbursement to the patient from third-party payers, such as governmental and private insurance plans. These third-party payers frequently require companies to provide predetermined discounts from list prices, and they are increasingly challenging the prices charged for pharmaceuticals and other medical products. Our products may not be considered cost-effective, and reimbursement to the patient may not be available or sufficient to allow us or our partners to sell our products on a competitive basis. It may not be possible to negotiate favorable reimbursement rates for our products.

In addition, the continuing efforts of third-party payers to contain or reduce the costs of healthcare through various means may limit our commercial opportunity and reduce any associated revenue and profits. We expect proposals to implement similar government control to continue. In addition, increasing emphasis on managed care will continue to put pressure on the pricing of pharmaceutical and biopharmaceutical products. Cost control initiatives could decrease the price that we or any current or potential collaborators could receive for any of our products and could adversely affect our profitability. In addition, in the U.S., in Canada and in many other countries, pricing and/or profitability of some or all prescription pharmaceuticals and biopharmaceuticals are subject to government control.

If we fail to obtain acceptable prices or an adequate level of reimbursement for our products, the sales of our products would be adversely affected or there may be no commercially viable market for our products.

Competition in our targeted markets is intense, and development by other companies could render our products or technologies non-competitive.

The biopharmaceutical field is highly competitive. New products developed by other companies in the industry could render our products or technologies non-competitive. Competitors are developing and testing products and technologies that would compete with the products that we are developing. Some of these products may be more

effective or have an entirely different approach or means of accomplishing the desired effect than our products. We expect competition from pharmaceutical and biopharmaceutical companies and academic research institutions to continue to increase over time. Many of our competitors and potential competitors have substantially greater product development capabilities and financial, scientific, marketing and

human resources than we do. Our competitors may succeed in developing products earlier and in obtaining regulatory approvals and patent protection for such products more rapidly than we can or at a lower price.

We may not obtain adequate protection for our products through our intellectual property.

We rely heavily on our proprietary information in developing and manufacturing our product candidates. Our success depends, in large part, on our ability to protect our competitive position through patents, trade secrets, trademarks and other intellectual property rights. The patent positions of pharmaceutical and biopharmaceutical firms, including us, are uncertain and involve complex questions of law and fact for which important legal issues remain unresolved. Applications for patents and trademarks in Canada, the U.S. and in other foreign territories have been filed and are being actively pursued by us. Pending patent applications may not result in the issuance of patents and we may not be able to obtain additional issued patents relating to our technology or products. Even if issued, patents to us or our licensing partners may be challenged, narrowed, invalidated, held to be unenforceable or circumvented, which could limit our ability to stop competitors from marketing similar products or limit the length of term of patent protection we may have for our products. Changes in either patent laws or in interpretations of patent laws in the U.S. and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection. The patents issued or to be issued to us may not provide us with any competitive advantage or protect us against competitors with similar technology. In addition, it is possible that third parties with products that are very similar to ours will circumvent our patents by means of alternate designs or processes. We may have to rely on method of use and new formulation protection for our compounds in development, and any resulting products, which may not confer the same protection as claims to compounds per se.

In addition, our patents may be challenged by third parties in patent litigation, which is becoming widespread in the biopharmaceutical industry. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There may also be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that our patents would, if challenged, be held by a court to be valid or enforceable or that a competitor's technology or product would be found by a court to infringe our patents. Our granted patents could also be challenged and revoked in U.S. post-grant proceedings as well as in opposition or nullity proceedings in certain countries outside the U.S. In addition, we may be required to disclaim part of the term of certain patents.

Patent applications relating to or affecting our business have been filed by a number of pharmaceutical and biopharmaceutical companies and academic institutions. A number of the technologies in these applications or patents may conflict with our technologies, patents or patent applications, and any such conflict could reduce the scope of patent protection which we could otherwise obtain. Because patent applications in the U.S. and many other jurisdictions are typically not published until eighteen months after their first effective filing date, or in some cases not at all, and because publications of discoveries in the scientific literature often lag behind actual discoveries, neither we nor our licensing partners can be certain that we or they were the first to make the inventions claimed in our or their issued patents or pending patent applications, or that we or they were the first to file for protection of the inventions set forth in these patent applications. If a third party has also filed a patent application in the U.S. covering our product candidates or a similar invention, we may have to participate in adversarial proceedings, such as interferences and deviation proceedings, before the United States Patent and Trademark Office to determine which party is entitled to a U.S. patent claiming the disputed invention. The costs of these proceedings could be substantial and it is possible that our efforts could be unsuccessful, resulting in a loss of our U.S. patent position.

In addition to patent protection, we may utilize orphan drug regulations, pediatric exclusivity or other provisions of the United States Food, Drug and Cosmetic Act of 1938, as amended, such as new chemical entity exclusivity or new formulation exclusivity, to provide market exclusivity for a drug candidate. Orphan drug regulations provide incentives to pharmaceutical and biotechnology companies to develop and manufacture drugs for the treatment of rare diseases, currently defined as diseases that exist in fewer than 200,000 individuals in the U.S., or, diseases that affect more than 200,000 individuals in the U.S. but that the sponsor does not realistically anticipate will generate a net profit. Under these provisions, a manufacturer of a designated orphan drug can seek tax benefits, and the holder of the first FDA approval of a designated orphan product will be granted a seven-year period of marketing exclusivity for

such FDA-approved orphan product. In the U.S., the FDA has the authority to grant additional data protection for approved drugs where the sponsor conducts specified testing in pediatric or adolescent populations. If granted, this pediatric exclusivity provides an additional six months which are added to the term of data protection as well as to the term of any relevant patents, to the extent these protections have not already expired. We may also seek to utilize market exclusivities in other territories, such as in the European Union (the "EU"). We cannot assure that any of our drug candidates will obtain such orphan drug designation, pediatric exclusivity, new chemical entity exclusivity or any other market exclusivity in the U.S., the EU or any other territory, or that we will be the first to receive the respective regulatory approval for such drugs so as to be eligible for any market exclusivity protection.

We also rely on trade secrets and proprietary know-how to protect our intellectual property. If we are unable to protect the confidentiality of our proprietary information and know-how, the value of our technology and products could be adversely affected. We seek to protect our unpatented proprietary information in part by requiring our employees, consultants, outside scientific collaborators and sponsored researchers and other advisors to enter into confidentiality agreements. These agreements provide that all confidential information developed or made known to the individual during the course of the individual's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. In the case of our employees, the agreements provide that all of the technology which is conceived by the individual during the course of employment is our exclusive property. These agreements may not provide meaningful protection or adequate remedies in the event of unauthorized use or disclosure of our proprietary information. In addition, it is possible that third parties could independently develop proprietary information and techniques substantially similar to ours or otherwise gain access to our trade secrets. If we are unable to protect the confidentiality of our proprietary information and know-how, competitors may be able to use this information to develop products that compete with our products and technologies, which could adversely impact our business.

We currently have the right to use certain patents and technologies under license agreements with third parties. Our failure to comply with the requirements of material license agreements could result in the termination of such agreements, which could cause us to terminate the related development program and cause a complete loss of our investment in that program.

As a result of the foregoing factors, we may not be able to rely on our intellectual property to protect our products in the marketplace.

We may infringe the intellectual property rights of others.

Our commercial success depends significantly on our ability to operate without infringing the patents and other intellectual property rights of third parties. There could be issued patents of which we are not aware that our products or methods may be found to infringe, or patents of which we are aware and believe we do not infringe but which we may ultimately be found to infringe. Moreover, patent applications and their underlying discoveries are in some cases maintained in secrecy until patents are issued. Because patents can take many years to issue, there may be currently pending applications of which we are unaware that may later result in issued patents that our products or technologies are found to infringe. Moreover, there may be published pending applications that do not currently include a claim covering our products or technologies but which nonetheless provide support for a later drafted claim that, if issued, our products or technologies could be found to infringe.

If we infringe or are alleged to infringe intellectual property rights of third parties, it will adversely affect our business. Our research, development and commercialization activities, as well as any product candidates or products resulting from these activities, may infringe or be accused of infringing one or more claims of an issued patent or may fall within the scope of one or more claims in a published patent application that may subsequently be issued and to which we do not hold a license or other rights. Third parties may own or control these patents or patent applications in the U.S. and abroad. These third parties could bring claims against us or our collaborators that would cause us to incur substantial expenses and, if successful against us, could cause us to pay substantial damages. Further, if a patent infringement suit were brought against us or our collaborators, we or they could be forced to stop or delay research, development, manufacturing or sales of the product or product candidate that is the subject of the suit.

The biopharmaceutical industry has produced a proliferation of patents, and it is not always clear to industry participants, including us, which patents cover various types of products. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. In the event of infringement or violation of another party's patent or other intellectual property rights, we may not be able to enter into licensing arrangements or make other arrangements at a reasonable cost. Any inability to secure licenses or alternative technology could result in delays in the introduction of our products or lead to prohibition of the manufacture or sale of products by us or our partners and collaborators.

Patent litigation is costly and time consuming and may subject us to liabilities.

Our involvement in any patent litigation, interference, opposition or other administrative proceedings will likely cause us to incur substantial expenses, and the efforts of our technical and management personnel will be significantly

diverted. In addition, an adverse determination in litigation could subject us to significant liabilities.

We may not obtain trademark registrations for our product candidates.

We have filed applications for trademark registrations in connection with our product candidates in various jurisdictions, including the U.S. We intend to file further applications for other possible trademarks for our product candidates. No assurance can be given that any of our trademark applications will be registered in the U.S. or elsewhere, or that the use of any registered or unregistered trademarks will confer a competitive advantage in the marketplace. Furthermore, even if we are successful in our trademark registrations, the FDA and regulatory authorities in other countries have their own process for drug nomenclature and their own views concerning appropriate proprietary names. The FDA and other regulatory authorities also have the power, even after granting market approval, to request a company to reconsider the name for a product because of evidence of confusion in the marketplace. No assurance can be given that the FDA or any other regulatory authority will approve of any of our trademarks or will not request reconsideration of one of our trademarks at some time in the future. The loss, abandonment, or cancellation of any of our trademarks or trademark applications could negatively affect the success of the product candidates to which they relate.

Our revenues and expenses may fluctuate significantly, and any failure to meet financial expectations may disappoint securities analysts or investors and result in a decline in the price of our securities.

We have a history of operating losses. Our revenues and expenses have fluctuated in the past and may continue to do so in the future. These fluctuations could cause our share price to decline. Some of the factors that could cause our revenues and expenses to fluctuate include but are not limited to:

- the inability to complete product development in a timely manner that results in a failure or delay in receiving the required regulatory approvals to commercialize our product candidates;
- the timing of regulatory submissions and approvals;
- the timing and willingness of any current or future collaborators to invest the resources necessary to commercialize our product candidates;
- the revenue available from royalties derived from our strategic partners;
- the nature and timing of licensing fees revenues;
- the nature and timing of tax credits and grants (R&D);
- the outcome of litigation, if any;
- changes in foreign currency fluctuations;
- the timing of achievement and the receipt of milestone payments from current or future collaborators; and
- failure to enter into new or the expiration or termination of current agreements with collaborators.

Due to fluctuations in our revenues and expenses, we believe that period-to-period comparisons of our results of operations are not necessarily indicative of our future performance. It is possible that in some future quarter or quarters, our revenues and expenses will be above or below the expectations of securities analysts or investors. In this case, the price of our securities could fluctuate significantly or decline.

We are currently dependent on certain strategic partners and may enter into future collaborations for the R&D of our product candidates.

We are currently dependent on certain strategic partners and may enter into future collaborations for the R&D of our product candidates. Our arrangements with these strategic partners may not provide us with the benefits we expect and may expose us to a number of risks.

We are dependent on, and rely upon, strategic partners to perform various functions related to our business, including, but not limited to, the R&D of some of our product candidates. Our reliance on these relationships poses a number of risks.

We may not realize the contemplated benefits of such agreements nor can we be certain that any of these parties will fulfill their obligations in a manner which maximizes our revenue. These arrangements may also require us to transfer certain material rights or issue our equity, voting or other securities to corporate partners, licensees and others. Any license or sublicense of our commercial rights may reduce our product revenue.

These agreements also create certain risks. The occurrence of any of the following or other events may delay product development or impair commercialization of our products:

not all of our strategic partners are contractually prohibited from developing or commercializing, either alone or with others, products and services that are similar to or competitive with our product candidates and, with respect to our strategic partnership agreements that do contain such contractual prohibitions or restrictions, prohibitions or restrictions do not always apply to our partners' affiliates and they may elect to pursue the development of any additional product

candidates and pursue technologies or products either on their own or in collaboration with other parties, including our competitors, whose technologies or products may be competitive with ours;

- our strategic partners may under-fund or fail to commit sufficient resources to marketing, distribution or other development of our products;
- we may not be able to renew such agreements;
- our strategic partners may not properly maintain or defend certain intellectual property rights that may be important to the commercialization of our products;
- our strategic partners may encounter conflicts of interest, changes in business strategy or other issues which could adversely affect their willingness or ability to fulfill their obligations to us (for example, pharmaceutical companies historically have re-evaluated their priorities following mergers and consolidations, which have been common in recent years in this industry);
- delays in, or failures to achieve, scale-up to commercial quantities, or changes to current raw material suppliers or product manufacturers (whether the change is attributable to us or the supplier or manufacturer) could delay clinical studies, regulatory submissions and commercialization of our product candidates; and
- disputes may arise between us and our strategic partners that could result in the delay or termination of the development or commercialization of our product candidates, resulting in litigation or arbitration that could be time-consuming and expensive, or causing our strategic partners to act in their own self-interest and not in our interest or those of our shareholders or other stakeholders.

In addition, our strategic partners can terminate our agreements with them for a number of reasons based on the terms of the individual agreements that we have entered into with them. If one or more of these agreements were to be terminated, we would be required to devote additional resources to developing and commercializing our product candidates, seek a new partner or abandon this product candidate which would likely cause a drop in the price of our securities.

We have entered into important strategic partnership agreements relating to certain of our product candidates for various indications. Detailed information on our research and collaboration agreements is available in our various reports and disclosure documents filed with the Canadian securities regulatory authorities and filed with or furnished to the United States Securities and Exchange Commission ("SEC"), including the documents incorporated by reference into this annual report on Form 20-F. For example, on April 10, 2013, we announced that we had entered into a co-development and profit-sharing agreement with Ergomed Clinical Research Ltd. ("Ergomed") for zoptarelin doxorubicin in endometrial cancer. Ergomed was selected as the contract clinical development organization to conduct the multicenter, multinational, randomized Phase 3 "ZoptEC" (Zoptarelin doxorubicin in Endometrial Cancer) trial with zoptarelin doxorubicin in endometrial cancer. Under the terms of this agreement, Ergomed will assume 30% (up to $10 million) of the clinical and regulatory costs for our Phase 3 ZoptEC trial of zoptarelin doxorubicin in endometrial cancer, which are currently estimated at approximately $30 million over the course of the study, and Ergomed will receive its return on investment based on an agreed single digit percentage of any net income received by us for zoptarelin doxorubicin in this indication, up to a specified maximum amount.

We have also entered into a variety of collaboration agreements with various universities and institutes under which we are obligated to support some of the research expenses incurred by the university laboratories and pay royalties on future sales of the products. In turn, we have retained exclusive rights for the worldwide exploitation of results generated during the collaborations.

We rely on third parties to conduct, supervise and monitor our clinical trials, and those third parties may not perform satisfactorily.

We rely on third parties such as CROs, medical institutions and clinical investigators to enroll qualified patients and conduct, supervise and monitor our clinical trials. Our reliance on these third parties for clinical development activities reduces our control over these activities. Our reliance on these third parties, however, does not relieve us of our regulatory responsibilities, including ensuring that our clinical trials are conducted in accordance with Good Clinical Practice guidelines and the investigational plan and protocols contained in an Investigational New Drug ("IND") application, or a comparable foreign regulatory submission. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. In addition, they may not complete activities

on schedule, or may not conduct our preclinical studies or clinical trials in accordance with regulatory requirements or our trial design. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, our efforts to obtain regulatory approvals for, and commercialize, our product candidates may be delayed or prevented.

In carrying out our operations, we are dependent on a stable and consistent supply of ingredients and raw materials. There can be no assurance that we, our contract manufacturers or our partners, will be able, in the future, to continue to purchase products from our current suppliers or any other supplier on terms similar to current terms or at all. An interruption in the availability of certain raw materials or ingredients, or significant increases in the prices paid by us for them, could have a material adverse effect on our business, financial condition, liquidity and operating results.
The failure to perform satisfactorily by third parties upon which we rely to manufacture and supply products may lead to supply shortfalls.
We will rely on third parties to manufacture and supply marketed products. We also have certain supply obligations vis à vis our licensing partners who are responsible for the marketing of the products. To be successful, our products have to be manufactured in commercial quantities in compliance with quality controls and regulatory requirements. Even though it is our objective to minimize such risk by introducing alternative suppliers to ensure a constant supply at all times, we cannot guarantee that we will not experience supply shortfalls and, in such event, we may not be able to perform our obligations under contracts with our partners.
We are subject to intense competition for our skilled personnel, and the loss of key personnel or the inability to attract additional personnel could impair our ability to conduct our operations.
We are highly dependent on our management and our clinical, regulatory and scientific staff, the loss of whose services might adversely impact our ability to achieve our objectives. Recruiting and retaining qualified management and clinical, scientific and regulatory personnel is critical to our success. Competition for skilled personnel is intense, and our ability to attract and retain qualified personnel may be affected by such competition.
Our strategic partners' manufacturing capabilities may not be adequate to effectively commercialize our product candidates.
Our manufacturing experience to date with respect to our product candidates consists of producing drug substance for clinical studies. To be successful, these product candidates have to be manufactured in commercial quantities in compliance with regulatory requirements and at acceptable costs. Our strategic partners' current manufacturing facilities have the capacity to produce projected product requirements for the foreseeable future, but we will need to increase capacity if expected sales grow more than originally forecasted. Our strategic partners may not be able to expand capacity or to produce additional product requirements on favorable terms. Moreover, delays associated with securing additional manufacturing capacity may reduce our revenues and adversely affect our business and financial position. There can be no assurance that we will be able to meet increased demand over time.
We are subject to the risk of product liability claims, for which we may not have or be able to obtain adequate insurance coverage.
The sale and use of our products, in particular our biopharmaceutical products, involve the risk of product liability claims and associated adverse publicity. Our risks relate to human participants in our clinical trials, who may suffer unintended consequences, as well as products on the market whereby claims might be made directly by patients, healthcare providers or pharmaceutical companies or others selling, buying or using our products. We manage our liability risks by means of insurance. We maintain liability insurance covering our liability for our preclinical and clinical studies and for our pharmaceutical products already marketed. However, we may not have or be able to obtain or maintain sufficient and affordable insurance coverage, including coverage for potentially very significant legal expenses, and without sufficient coverage any claim brought against us could have a materially adverse effect on our business, financial condition or results of operations.
Our business involves the use of hazardous materials which requires us to comply with environmental and occupational safety laws regulating the use of such materials. If we violate these laws, we could be subject to significant fines, liabilities or other adverse consequences.
Our discovery and development processes involve the controlled use of hazardous and radioactive materials. We are subject to federal, provincial and local laws and regulations governing the use, manufacture, storage, handling and disposal of such materials and certain waste products. The risk of accidental contamination or injury from these materials cannot be completely eliminated. In the event of an accident or a failure to comply with environmental or occupational safety laws, we could be held liable for any damages that result, and any such liability could exceed our resources. We may not be adequately insured against this type of liability. We may be required to incur significant

costs to comply with environmental laws and regulations in the future, and our operations, business or assets may be materially adversely affected by current or future environmental laws or regulations.

We are a holding company, and claims of creditors of our subsidiaries will generally have priority as to the assets of such subsidiaries over our claims and those of our creditors and shareholders.

Aeterna Zentaris Inc. is a holding company and a substantial portion of our assets is the share capital of our subsidiaries. AEZS GmbH, our principal operating subsidiary, based in Frankfurt, Germany, holds most of our intellectual property rights, which represent the principal assets of our business.

Because Aeterna Zentaris Inc. is a holding company, our obligations to our creditors are structurally subordinated to all existing and future liabilities of our subsidiaries. Therefore, our rights and the rights of our creditors to participate in any distribution of the assets of any subsidiary in the event that such subsidiary were to be liquidated or reorganized or in the event of any bankruptcy or insolvency proceeding relating to or involving such subsidiary, and therefore the rights of the holders of our Common Shares to participate in those assets, are subject to the prior claims of such subsidiary's creditors. To the extent that we may be a creditor with recognized claims against any such subsidiary, our claims would still be subject to the prior claims of our subsidiary's creditors to the extent that they are secured or senior to those held by us.

Holders of our Common Shares are not creditors of our subsidiaries. Claims to the assets of our subsidiaries will derive from our own ownership interest in those operating subsidiaries. Claims of our subsidiaries' creditors will generally have priority as to the assets of such subsidiaries over our own ownership interest claims and will therefore have priority over the holders of our Common Shares. Our subsidiaries' creditors may from time to time include general creditors, trade creditors, employees, secured creditors, taxing authorities, and creditors holding guarantees. Accordingly, in the event of any foreclosure, dissolution, winding-up, liquidation or reorganization, or a bankruptcy or insolvency proceeding relating to us or our property, or any subsidiary, there can be no assurance as to the value, if any, that would be available to holders of our Common Shares.

In addition, any distributions to us by our subsidiaries could be subject to monetary transfer restrictions in the jurisdictions in which our subsidiaries operate.

Our subsidiaries may incur additional indebtedness and other liabilities.

It may be difficult for U.S. investors to obtain and enforce judgments against us because of our Canadian incorporation and German presence.

We are a company existing under the laws of Canada. Many of our directors and officers, and certain of the experts named herein, are residents of Canada or otherwise reside outside the U.S., and all or a substantial portion of their assets, and a substantial portion of our assets, are located outside the U.S. Consequently, although we have appointed an agent for service of process in the U.S., it may be difficult for investors in the U.S. to bring an action against such directors, officers or experts or to enforce against those persons or us a judgment obtained in a U.S. court predicated upon the civil liability provisions of federal securities laws or other laws of the U.S. Investors should not assume that foreign courts (1) would enforce judgments of U.S. courts obtained in actions against us or such directors, officers or experts predicated upon the civil liability provisions of the U.S. federal securities laws or the securities or "blue sky" laws of any state within the U.S. or (2) would enforce, in original actions, liabilities against us or such directors, officers or experts predicated upon the U.S. federal securities laws or any such state securities or "blue sky" laws. In addition, we have been advised by our Canadian counsel that in normal circumstances, only civil judgments and not other rights arising from U.S. securities legislation (for example, penal or similar awards made by a court in a regulatory prosecution or proceeding) are enforceable in Canada and that the protections afforded by Canadian securities laws may not be available to investors in the U.S.

Health care reform measures could hinder or prevent the commercial success of our product candidates and adversely affect our business.

The business prospects and financial condition of pharmaceutical and biotechnology companies are affected by the efforts of governmental and third-party payers to contain or reduce the costs of health care. In the U.S. and in foreign jurisdictions there have been, and we expect that there will continue to be, a number of legislative and regulatory proposals aimed at changing the health care system, such as proposals relating to the pricing of healthcare products and services in the U.S. or internationally, the reimportation of drugs into the U.S. from other countries (where they are then sold at a lower price), and the amount of reimbursement available from governmental agencies or other third party payers. For example, drug manufacturers are required to have a national rebate agreement with the Department

of Health and Human Services in order to obtain state Medicaid coverage, which requires manufacturers to pay a rebate on drugs dispensed to Medicaid patients. On January 27, 2012, the Centers for Medicare and Medicaid Services ("CMS") issued a proposed regulation covering the calculation of Average Manufacturer Price ("AMP") which is the key variable in the calculation of these rebates.

In March 2010, President Obama signed into law a legislative overhaul of the U.S. healthcare system, known as the Patient Protection and Affordable Care Act of 2010, as amended by the Healthcare and Education Affordability Reconciliation Act of 2010 (collectively, the "PPACA"), which may have far-reaching consequences for most healthcare companies, including specialty biopharmaceutical companies like us. For example, if reimbursement for our product candidates is substantially less than we expect, our revenue prospects could be materially and adversely impacted.

Regardless of the impact of the PPACA on us, the U.S. government and other governments have shown significant interest in pursuing healthcare reform and reducing healthcare costs. Any government-adopted reform measures could cause significant pressure on the pricing of healthcare products and services, including our product candidates, in the United States and internationally, as well as the amount of reimbursement available from governmental agencies and other third-party payors.

In addition, on September 27, 2007, the Food and Drug Administration Amendments Act of 2007 was enacted, giving the FDA enhanced post-market authority, including the authority to require post-marketing studies and clinical trials, labeling changes based on new safety information, and compliance with risk evaluations and mitigation strategies approved by the FDA. The FDA's exercise of this authority may result in delays or increased costs during the period of product development, clinical trials and regulatory review and approval, which may also increase costs related to complying with new post-approval regulatory requirements, and increase potential FDA restrictions on the sale or distribution of approved products.

We are subject to additional reporting requirements under applicable Canadian securities laws and the Sarbanes-Oxley Act in the U.S.. We can provide no assurance that we will at all times in the future be able to report that our internal controls over financial reporting are effective.

As a public company, we are required to comply with Section 404 of the U.S. Sarbanes-Oxley Act ("Section 404") and National Instrument 52-109 - Certification of Disclosure in Issuers' Annual and Interim Filings, and we are required to obtain an annual attestation from our independent auditors regarding our internal control over financial reporting. In any given year, we cannot be certain as to the time of completion of our internal control evaluation, testing and remediation actions or of their impact on our operations. Upon completion of this process, we may identify control deficiencies of varying degrees of severity under applicable SEC and Public Company Accounting Oversight Board rules and regulations. As a public company, we are required to report, among other things, control deficiencies that constitute material weaknesses or changes in internal controls that, or that are reasonably likely to, materially affect internal controls over financial reporting. A "material weakness" is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the Company's annual consolidated financial statements will not be prevented or detected on a timely basis. If we fail to comply with the requirements of Section 404, Canadian requirements or report a material weakness, we might be subject to regulatory sanction and investors may lose confidence in our consolidated financial statements, which may be inaccurate if we fail to remedy such material weakness.

It is possible that we may be a passive foreign investment company, which could result in adverse tax consequences to U.S. investors.

Adverse U.S. federal income tax rules apply to "U.S. Holders" (as defined in "Item 10.E – Taxation – Certain Material U.S. Federal Income Tax Considerations" in this annual report on Form 20-F) that directly or indirectly hold Common Shares of a passive foreign investment company ("PFIC"). We will be classified as a PFIC for U.S. federal income tax purposes for a taxable year if (i) at least 75% of our gross income is “passive income” or (ii) at least 50% of the average value of our assets, including goodwill (based on annual quarterly average), is attributable to assets which produce passive income or are held for the production of passive income.

We believe that we were not a PFIC for the 2013 taxable year. However, the PFIC determination depends on the application of complex U.S. federal income tax rules concerning the classification of our assets and income for this purpose, and these rules are uncertain in some respects. In addition, the fair market value of our assets may be determined in large part by the market price of our Common Shares, which is likely to fluctuate, and the composition of our income and assets will be affected by how, and how quickly, we spend any cash that is raised in any financing transaction. No assurance can be provided that we will not be classified as a PFIC for the 2014 taxable year and for

any future taxable year.

PFIC characterization could result in adverse U.S. federal income tax consequences to U.S. Holders. In particular, absent certain elections, a U.S. Holder would generally be subject to U.S. federal income tax at ordinary income tax rates, plus a possible interest charge, in respect of a gain derived from a disposition of our Common Shares, as well as certain distributions by us. If we are treated as a PFIC for any taxable year, a U.S. Holder may be able to make an election to "mark to market" Common Shares each taxable year and recognize ordinary income pursuant to such election based upon increases in the value of the Common Shares. In addition, U.S. Holders may mitigate the adverse tax consequences of the PFIC rules by making a "qualified electing fund" ("QEF") election; however, the Company does not expect to provide the information regarding its income that would be necessary for a U.S. Holder to make a QEF election.

If the Company is a PFIC, U.S. Holders will generally be required to file an annual information return with the Internal Revenue Service (the "IRS") (on IRS Form 8621, which PFIC shareholders will be required to file with their U.S. federal income tax or information returns) relating to their ownership of Common Shares. This new filing requirement is in addition to any preexisting reporting requirements that apply to a U.S. Holder's interest in a PFIC (which this requirement does not affect).

For a more detailed discussion of the potential tax impact of us being a PFIC, see "Item 10.E – Taxation – Certain Material U.S. Federal Income Tax Considerations" in this annual report on Form 20-F. The PFIC rules are complex. U.S. Holders should consult their tax advisors regarding the potential application of the PFIC regime and any reporting obligations to which they may be subject under that regime.

We may incur losses associated with foreign currency fluctuations.

Our operations are in many instances conducted in currencies other than the euro, our functional currency. Fluctuations in the value of currencies could cause us to incur currency exchange losses. We do not currently employ a hedging strategy against exchange rate risk. We cannot assert with any assurance that we will not suffer losses as a result of unfavorable fluctuations in the exchange rates between the US dollar, the euro, the Canadian dollar and other currencies. For more information, see "Item 11. – Quantitative and Qualitative Disclosures About Market Risk" in this annual report on Form 20-F.

Legislative actions, new accounting pronouncements and higher insurance costs are likely to impact our future financial position or results of operations.

Changes in financial accounting standards or implementation of accounting standards may cause adverse, unexpected revenue or expense fluctuations and affect our financial position or results of operations. New pronouncements and varying interpretations of pronouncements have occurred with greater frequency and are expected to occur in the future, and we may make or be required to make changes in our accounting policies in the future. Compliance with changing regulations of corporate governance and public disclosure, notably with respect to internal controls over financial reporting, may result in additional expenses. Changing laws, regulations and standards relating to corporate governance and public disclosure are creating uncertainty for companies such as ours, and insurance costs are increasing as a result of this uncertainty.

The outcome of any future claims and litigation could have a material adverse impact on our business, financial condition and results of operations.

The Company and its subsidiaries may, from time to time, be parties to litigation in the normal course of business. Due to the inherent uncertainties of litigation, it is not possible to predict the final outcome of these lawsuits or determine the amount of any potential losses, if any, and we may, in the future, be subject litigation proceedings, including class action lawsuits. In the event we are required or determine to pay amounts in connection with any such lawsuits, such amounts could be significant and could have a material adverse impact on our liquidity, business, financial condition and results of operations.

Risks Relating to our Common Shares

Our share price is volatile, which may result from factors outside of our control. If our Common Shares were to be delisted from NASDAQ Capital Market ("NASDAQ") or Toronto Stock Exchange (the "TSX"), investors may have difficulty in disposing of our Common Shares held by them.

Our Common Shares are currently listed and traded only on NASDAQ and TSX. Our valuation and share price since the beginning of trading after our initial listings, first in Canada and then in the U.S., have had no meaningful relationship to current or historical financial results, asset values, book value or many other criteria based on conventional measures of the value of shares.

Between January 1, 2013 and December 31, 2013, the closing price of our Common Shares ranged from $1.03 to $3.23 on NASDAQ and from C$1.08 to C$3.27 per share on TSX. Our share price may be affected by developments directly affecting our business and by developments out of our control or unrelated to us. The stock market generally, and the biopharmaceutical sector in particular, are vulnerable to abrupt changes in investor sentiment. Prices of shares and trading volume of companies in the biopharmaceutical industry can swing dramatically in ways unrelated to, or that bear a disproportionate relationship to, operating performance. Our share price and trading volume may fluctuate based on a number of factors including, but not limited to:

- clinical and regulatory developments regarding our product candidates;
- delays in our anticipated development or commercialization timelines;
- developments regarding current or future third-party collaborators;
- other announcements by us regarding technological, product development or other matters;
- arrivals or departures of key personnel;

- governmental or regulatory action affecting our product candidates and our competitors' products in the U.S., Canada and other countries;
- developments or disputes concerning patent or proprietary rights;
- actual or anticipated fluctuations in our revenues or expenses;
- general market conditions and fluctuations for the emerging growth and biopharmaceutical market sectors; and
- economic conditions in the U.S., Canada or abroad.

Our listing on both NASDAQ and TSX may increase price volatility due to various factors, including different ability to buy or sell our Common Shares, different market conditions in different capital markets and different trading volumes. In addition, low trading volume may increase the price volatility of our Common Shares. A thin trading market could cause the price of our Common Shares to fluctuate significantly more than the stock market as a whole. A period of large price decline in our Common Shares could increase the risk that securities class action litigation could be initiated against us. Litigation of this type and other litigation could result in substantial costs and diversion of management's attention and resources, which would adversely affect our business. Any adverse determination in litigation could also subject us to significant liabilities.

We must meet continuing listing requirements to maintain the listing of our Common Shares on NASDAQ and TSX. For continued listing, NASDAQ requires, among other things, that listed securities maintain a minimum closing bid price of not less than $1.00 per share.

If our Common Shares trade for 30 consecutive business days below the required $1.00 minimum closing bid price, we expect that NASDAQ would then send us a deficiency notice and provide us with a period of 180 calendar days to regain compliance with the minimum bid price requirement. In order to regain compliance, the closing bid price of our Common Shares would have to be at least US$1.00 for a minimum of 10 consecutive business days. If we were not able to regain compliance, NASDAQ would notify us that our securities are subject to delisting. At that time, we could appeal the determination to delist our securities to a Listing Qualifications Panel.

In addition to the minimum bid price requirement, the continued listing rules of NASDAQ require us to meet at least one of the following listing standards: (i) stockholders' equity of at least $2.5 million (the "Equity Standard"), (ii) market value of listed securities (calculated by multiplying the daily closing bid price of our Common Shares by our total outstanding Common Shares) of at least $35 million (the "Market Value Standard") or (iii) net income from continuing operations (in the latest fiscal year or in two of the last three fiscal years) of at least $500,000 (the "Net Income Standard"). If our total market capitalization decreases to an amount less than $35 million for 30 consecutive trading days, it is possible that we could no longer meet any of these three listing standards. Similar to the process described above in the minimum bid price context, if we fail to meet the Market Value Standard for 30 consecutive trading days and do not otherwise meet the Equity Standard or the Net Income Standard, we expect that we would then receive a notification letter from NASDAQ advising us that we fail to comply with the Market Value Standard and providing us a period of 180 calendar days to regain compliance with the Market Value Standard. In order to regain compliance with the Market Value Standard, the market value of our listed securities would have to be at least $35 million for a period of 10 consecutive business days. Otherwise, our securities may then be subject to delisting.

There can be no assurance that our Common Shares will remain listed on NASDAQ. If we fail to meet any of NASDAQ's continued listing requirements, our Common Shares may be delisted. Any delisting of our Common Shares may adversely affect a shareholder's ability to dispose, or obtain quotations as to the market value, of such shares.

We do not intend to pay dividends in the near future.

To date, we have not declared or paid any dividends on our Common Shares. We currently intend to retain our future earnings, if any, to finance further research and the overall commercial expansion of our business. As a result, the return on an investment in our Common Shares will, for the foreseeable future, depend upon any future appreciation in value. There is no guarantee that our Common Shares will appreciate in value or even maintain the price at which shareholders have purchased them.

Future issuances of securities and hedging activities may depress the trading price of our Common Shares.

Any additional or future issuance of Convertible Securities, including the issuance of Common Shares upon the exercise of stock options and upon the exercise of outstanding warrants, could dilute the interests of our existing

shareholders, and could substantially decrease the trading price of our Common Shares. We may issue equity securities in the future for a number of reasons, including to finance our operations and business strategy, to satisfy our obligations upon the exercise of options or warrants or for other reasons. Our Stock Option Plan generally permits us to have outstanding, at any given time, stock options that are exercisable for a maximum number of Common Shares equal to 11.4% of all then issued and outstanding Common Shares. As at December 31, 2013, there were:

- 45,312,009 Common Shares issued and outstanding;
- no issued and outstanding preferred shares;
- 20,107,410 Common Shares issuable upon exercise of outstanding warrants; and
- 2,412,573 stock options outstanding.

In addition, the price of Common Shares could also be affected by possible sales of Common Shares by investors who view other investment vehicles as more attractive means of equity participation in us and by hedging or arbitrage trading activity that may develop involving our Common Shares. This hedging or arbitrage could, in turn, affect the trading price of our Common Shares.

Our articles of incorporation contain "blank check" preferred share provisions, which could delay or impede an acquisition of our company.

Our articles of incorporation, as amended, authorize the issuance of an unlimited number of "blank check" preferred shares, which could be issued by our Board of Directors without shareholder approval and may contain liquidation, dividend and other rights equivalent or superior to our Common Shares. In addition, we have implemented in our constating documents an advance notice procedure for shareholder approvals to be brought before an annual meeting of our shareholders, including proposed nominations of persons for election to our Board of Directors. These provisions, among others, whether alone or together, could delay or impede hostile takeovers and changes in control or changes in our management. Any provision of our constating documents that has the effect of delaying or deterring a change in control could limit the opportunity for our shareholders to receive a premium for their Common Shares and could also affect the price that some investors are willing to pay for our Common Shares.

Item 4. Information on the Company

A. History and development of the Company

We are a specialty biopharmaceutical Company engaged in developing novel treatments in oncology and endocrinology. Our pipeline encompasses compounds at various stages of development.

We were incorporated on September 12, 1990 under the Canada Business Corporations Act (the "CBCA") and continue to be governed by the CBCA. Our registered address and head office is located at 1405 du Parc-Technologique Blvd., Quebec City, Quebec, Canada G1P 4P5, our telephone number is (418) 652-8525 and our website is www.aezsinc.com. None of the documents or information found on our website shall be deemed to be included in or incorporated by reference into this annual report on Form 20-F, unless such document is specifically incorporated herein by reference.

On December 30, 2002, we acquired Zentaris AG, a biopharmaceutical company based in Frankfurt, Germany. Zentaris was a spin-off of Asta Medica GmbH, a former pharmaceutical company affiliated with Degussa AG.

In May 2004, we changed our name to Aeterna Zentaris Inc. and on May 11, 2007, Zentaris GmbH was renamed Aeterna Zentaris GmbH ("AEZS GmbH"). AEZS GmbH is our principal operating subsidiary.

On April 6, 2005, our former subsidiary, Atrium Biotechnologies Inc. (now Atrium Innovations Inc.) ("Atrium"), completed its initial public offering in Canada and began trading on the TSX under the ticker symbol "ATB".

In 2006, we spun off our ownership interest in Atrium in two phases. As of January 2, 2007 we no longer held any ownership interest in Atrium.

In May 2007, we opened an office in the United States, located at 20 Independence Boulevard, Warren, New Jersey 07059-2731. The Company moved this office to a new location in December 2011 at 25 Mountainview Blvd., Suite 203, Basking Ridge, NJ 07920.

On October 2, 2012, we effected a 6-to-1 Share Consolidation (reverse stock split). Our Common Shares commenced trading on a consolidated and adjusted basis on both NASDAQ and TSX on October 5, 2012.

On October 1, 2013, we announced the completion of our previously announced agreements with various partners and licensees with respect to the manufacturing rights and obligations for our Cetrotide® product. The principal outcome of such agreements is the transfer of all manufacturing rights and the grant of a license to a subsidiary of Merck KGaA of Darmstadt, Germany for the manufacture, testing, assembling, packaging, storage and release of Cetrotide® in all territories (the "Cetrotide® Business"). Following this transfer, the Cetrotide® Business has been presented in our consolidated financial statements as a discontinued operation.

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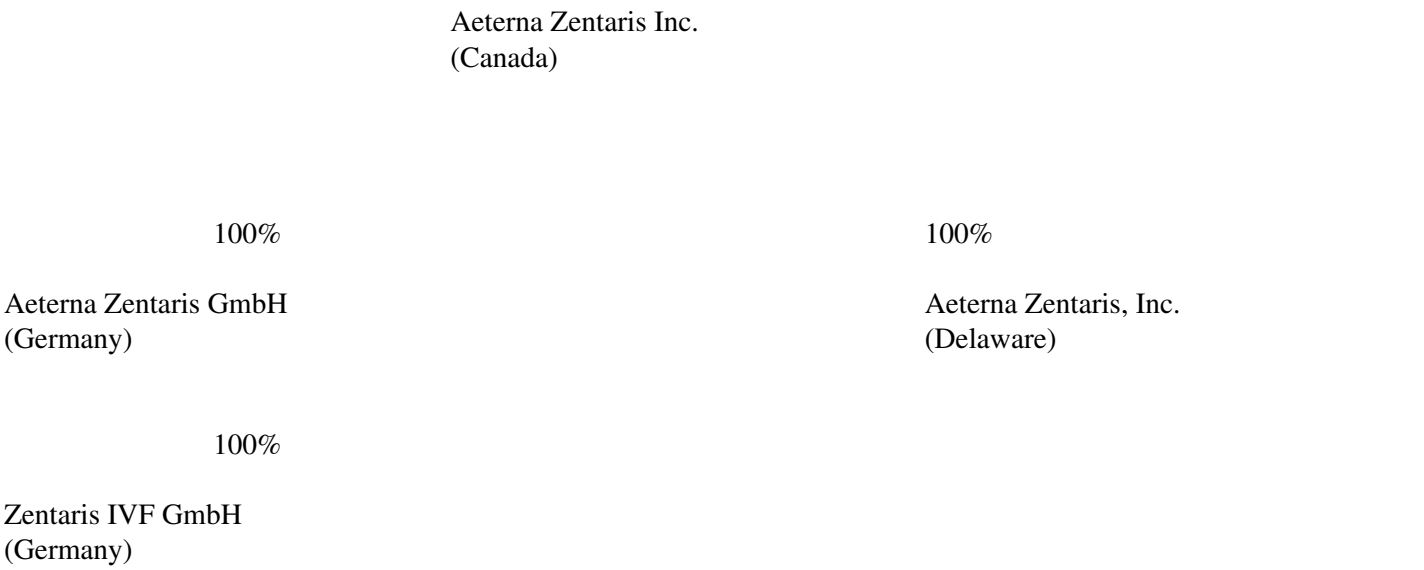
We currently have three wholly-owned direct and indirect subsidiaries, Aeterna Zentaris GmbH (Germany), based in Frankfurt, Germany, Zentaris IVF GmbH, a direct wholly-owned subsidiary of AEZS Germany based in Frankfurt, Germany, and Aeterna Zentaris, Inc., an entity incorporated in the State of Delaware with an office in Basking Ridge, New Jersey in the United States.



In oncology, our current principal focus is on our ongoing Phase 3 "ZoptEC" (Zoptarelin doxorubicin in Endometrial Cancer) trial in endometrial cancer with zoptarelin doxorubicin. In endocrinology, we are focused on preparing the launch of MACRILEN™ (macimorelin). This product is currently subject to a standard review by the FDA. If approved, MACRILEN™ will be the first orally administrated drug indicated for the evaluation of Adult Growth Hormone Deficiency ("AGHD") by evaluating the pituitary gland secretion of growth hormone in response to an oral dose of the product. We are also investigating various additional compounds as potential treatments for a host of unmet medical needs, as depicted in the chart reproduced under the heading, "Our Product Pipeline".

Our Common Shares are listed for trading on the TSX under the trading symbol "AEZ" and on NASDAQ under the trading symbol "AEZS".

The Company's agent for service of process and SEC matters in the United States is its wholly-owned subsidiary, Aeterna Zentaris, Inc., located at 25 Mountainview Blvd., Suite 203, Basking Ridge, NJ 07920.

There have been no public takeover offers by third parties with respect to the Company or by the Company in respect of other companies' shares during the last or current fiscal year.

B. Business overview

We are a specialty biopharmaceutical Company engaged in developing novel treatments in oncology and endocrinology. Our pipeline encompasses compounds at various stages of development.

Over the years, the Company has incurred recurring operating losses, having invested significantly in our R&D activities, as well as supporting our general and administrative expenses. We have financed our operations through different sources including the issuance of Common Shares and warrants, the conclusion of strategic alliances with licensee partners and R&D grants awarded by governmental agencies. The Company expects to continue to incur operating losses and may require significant capital to fulfill our future obligations. See the capital disclosures and the liquidity risk sections in "Item 5. – Operating and Financial Review and Prospects – Liquidity Risk".

In oncology, we have an ongoing Phase 3 ZoptEC trial in endometrial cancer under a Special Protocol Assessment ("SPA") with the FDA with zoptarelin doxorubicin, a doxorubicin Luteinizing Hormone-Releasing Hormone ("LHRH") targeted conjugate compound for which we have successfully completed a Phase 2 trial in advanced endometrial and advanced ovarian cancer. We are also advancing a Phase 2 investigator-driven trial with zoptarelin

doxorubicin in castration- and taxane-resistant prostate cancer. Our oncology pipeline also encompasses earlier-stage programs, including our AEZS-120, a targeted, live recombinant oral tumor vaccine candidate, our Erk/PI3K inhibitors, such as AEZS-129 and AEZS-136 and our disorazol Z

product candidates (AEZS-137 and AEZS-138). We are also investigating various additional compounds as potential treatments for a host of unmet medical needs.

In endocrinology, we have filed an NDA in the U.S. for the registration of MACRILEN™, our orally available peptidomimetic ghrelin receptor agonist with growth hormone secretagogue activity. On January 6, 2014, we announced that the FDA had accepted for substantive review our NDA for MACRILEN™. The acceptance for filing of the NDA indicates that the FDA has determined that the application is sufficiently complete to permit a substantive review. The NDA, submitted on November 5, 2013, seeks approval for the commercialization of MACRILEN™, which, if approved, will be the first orally administered drug indicated for the evaluation of AGHD by evaluating the pituitary gland secretion of growth hormone in response to an oral dose of the product. The application is subject to a standard review by the FDA.

Recent Developments

For a complete description of our recent corporate and pipeline developments, refer to "Item 5. – Operating and Financial Review and Prospects – Key Developments in 2013".

Our Business Strategy

Our primary business strategy is to pursue the successful development and commercialization of our pipeline with a focus on our principal product candidates zoptarelin doxorubicin and MACRILEN™ in oncology and endocrinology and achieve successful revenue-generating in-/out-licensing opportunities. Our vision is to become a growth-oriented specialty biopharmaceutical company.

Our product pipeline

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(1) Investigator-driven and sponsored.

(2) Phase 2 in ovarian cancer completed.

(3) Sponsored entirely by our licensee partners (Spectrum Pharmaceuticals, World (ex-Japan, Korea and other Asian countries) – Handok Pharmaceuticals, Korea and other Asian countries for benign prostatic hyperplasia ("BPH") indication – Nippon Kayaku, Japan for oncology indications).

(4) Sponsored entirely by our licensee partners (Yakult Honsha, Japan – Handok Pharmaceuticals, Korea – Hikma Pharmaceuticals, Middle East/North Africa).

Oncology

In oncology, we are conducting the ZoptEC Phase 3 study under a SPA with the FDA for zoptarelin doxorubicin in endometrial cancer. We are also advancing an investigator-driven Phase 2 trial with zoptarelin doxorubicin in castration- and taxane-resistant prostate cancer.

Zoptarelin doxorubicin

Zoptarelin doxorubicin represents a new targeting concept in oncology using a hybrid molecule composed of a synthetic peptide carrier and a well-known chemotherapy agent, doxorubicin. Zoptarelin doxorubicin is the first intravenous drug in advanced clinical development that directs the chemotherapy agent specifically to LHRH-receptor expressing tumors, resulting in more targeted treatment with less damage to healthy tissue. The product has successfully completed Phase 2 studies for the treatment of ovarian and endometrial cancer. We hold the worldwide rights to zoptarelin doxorubicin pursuant to an exclusive license agreement with Tulane University, as licensor, and AEZS GmbH, as licensee.

Endocrinology

In endocrinology, an NDA is under review by the FDA for the registration of MACRILEN™, for use in the evaluation of AGHD, in the U.S. Furthermore, macimorelin is being investigated in a Phase 2A trial in cancer-induced cachexia currently conducted under a cooperative R&D agreement ("CRADA") with the Michael E. DeBakey Veterans Affairs Medical Center that is funding the study. We hold the worldwide rights to macimorelin pursuant to an exclusive license agreement with The French Centre National de la Recherche Scientifique, as licensor, and AEZS GmbH, as licensee.

MACRILEN™

MACRILEN™ is an orally available peptidomimetic ghrelin receptor agonist with growth hormone secretagogue activity. MACRILEN™ has been granted orphan-drug designation by the FDA. On January 6, 2014, we announced that the FDA had accepted for substantive review our NDA for MACRILEN™ for the evaluation of AGHD. The application is subject to a standard review by the FDA.

Clinical and Preclinical Programs

Our oncology pipeline also encompasses other earlier-stage programs, including AEZS-120, a targeted, live recombinant oral tumor vaccine candidate, our Erk/PI3K inhibitors, including AEZS-129 and AEZS-136, as well as our disorazol Z product candidates comprise AEZS-137 and AEZS-138.

We are also investigating various additional compounds as potential treatments for a host of unmet medical need. We also continue to perform targeted drug discovery activities from which we are able to derive preclinical candidates. This drug discovery includes high throughput screening systems and a library of more than 120,000 compounds.

We are currently at a stage in which some of our products and product candidates are being further developed jointly with strategic partners or with funding from governmental organizations.

1.0 ONCOLOGY

1.1 TUMOR TARGETING CYTOTOXIC CONJUGATES AND CYTOTOXICS

Cytotoxic conjugates

In view of the non-specific toxicity of most chemotherapeutic agents against normal cells, targeting such drugs to cancerous tissue offers a potential benefit for patients with advanced or metastatic tumors. Targeted cytotoxic peptide conjugates are hybrid molecules composed of a cytotoxic moiety linked to a peptide carrier which binds to receptors on tumors. Cytotoxic conjugates are designed to achieve differential delivery, or targeting, of the cytotoxic agent to cancer vs. normal cells.

Our cytotoxic conjugates represent a novel oncological strategy to control and reduce toxicity and improve the effectiveness of cytotoxic drugs.

In zoptarelin doxorubicin, the most advanced of our cytotoxic conjugates, doxorubicin is chemically linked to an LHRH agonist, a modified natural hormone with affinity for the LHRH receptor. This design allows for the specific binding and selective uptake of the cytotoxic conjugate by LHRH receptor-positive tumors. Potential benefits of this targeted approach include a more favorable safety profile with lower incidence and severity of side effects, as normal tissues would be spared from the toxic effects of doxorubicin. In addition, the targeted approach may enable treatment of LHRH receptor-positive cancers that have become refractory to doxorubicin which has been administered in its non-targeted form.

1.1.1 Zoptarelin doxorubicin – Ovarian and Endometrial Cancer

In 2007, a Phase 2 open-label, non-comparative, multicenter two indication trial stratified with two stages Simon Design was prepared. The study was planned to involve up to 82 patients, with up to 41 patients each with a diagnosis of platinum-resistant ovarian cancer (stratum A) or disseminated endometrial cancer (stratum B). Under coordination by Prof. Günter Emons, M.D., Chairman of the Department of Obstetrics & Gynaecology at the University of Göttingen, Germany, this open-label, multicenter and multinational Phase 2 study "AGO-GYN 5" was conducted by the German AGO Study Group (Arbeitsgemeinschaft Gynäkologische Onkologie / Gynaecological Oncology Working Group), in cooperation with clinical sites in Europe. An i.v. infusion of zoptarelin doxorubicin (267 mg/m^2) was administered over a period of two hours, every Day 1 of a 21-day (3-week) cycle. The proposed duration of the study treatment was six cycles. The study was performed with 14 centers of the German Gynaecological Oncology Working Group, in cooperation with three clinical sites in Europe. The primary efficacy endpoint was a response rate with a success criterion at the end of Stage II defined as five or more patients with partial or complete tumor responses according to Response Evaluation Criteria in Solid Tumors ("RECIST") and/or Gynaecologic Cancer Intergroup ("GCIG") guidelines. Secondary endpoints included time to progression ("TTP"), survival, toxicity, as well as adverse effects. In October 2008, we announced that we had entered the second stage of patient recruitment for the Phase 2 trial in platinum-resistant ovarian cancer indication. This decision was taken following the report of two partial responses ("PR") among patients with ovarian cancer. The second stage of patient recruitment for the endometrial cancer indication was reached in November 2008 and was based on the report of one complete response ("CR") and two PR among 14 patients with endometrial cancer.

On November 2, 2009, we announced positive preliminary efficacy data for the Phase 2 study in patients with LHRH-receptor positive platinum-resistant and taxane-pretreated ovarian cancer. All 43 patients who had entered the study had completed their treatment, and a preliminary evaluation had shown that the study had met its predefined primary efficacy endpoint of five or more responders in 41 evaluable patients. Responders, as well as patients with stable disease after completion of treatment with zoptarelin doxorubicin, were to be followed to assess the duration of response and, ultimately, overall survival ("OS").

On November 24, 2009, we announced positive results for the Phase 2 study in patients with endometrial cancer. Preliminary evaluation showed that the study met its predefined primary efficacy endpoint of five or more responders in endometrial cancer patients. Responders, as well as patients with stable disease after completion of treatment with zoptarelin doxorubicin, were to be followed to assess the duration of progression free survival ("PFS") and, ultimately, OS.

On May 6, 2010, we announced that we had received orphan drug designation from the FDA for zoptarelin doxorubicin for the treatment of ovarian cancer.

On May 17, 2010, we announced that we had received a positive opinion for orphan medicinal product designation from the COMP of the EMA for zoptarelin doxorubicin for the treatment of ovarian cancer.

On June 7, 2010, Prof. Günter Emons, Chairman, Department of Obstetrics & Gynaecology Georg-August University Göttingen, Germany, presented positive efficacy and safety data for zoptarelin doxorubicin in ovarian cancer at the American Society of Clinical Oncology's ("ASCO") Annual Meeting. The poster (abstract #5035), was entitled "Phase 2 study of AEZS-108, a targeted cytotoxic LHRH analog, in patients with LHRH receptor-positive platinum resistant ovarian cancer".

42 patients with platinum-resistant ovarian cancer entered the study. Efficacy included PR in five patients (11.9%) and stable disease for more than twelve weeks in eleven patients (26.2%). Based on those data, a clinical benefit rate ("CBR") of 38% was estimated. Median TTP and OS were evaluated as 3.5 months (104 days) and 15.6 months (475 days), respectively. OS compared favourably with data from Doxil® and topotecan (8-9 months). In all, tolerability of zoptarelin doxorubicin was good and commonly allowed retreatment as scheduled. Only one patient (2.4%) had a dose reduction, and overall, 25 of 170 (14.7%) courses were given with a delay, including cases in which delay was not related to toxicity. Severe (Grade 3 or 4) toxicity was mainly restricted to rapidly reversible hematologic toxicity (leukopenia / neutropenia) associated with fever in three cases. Good tolerability of zoptarelin doxorubicin was also reflected with only a few patients with non-hematological toxicities of Grade 3 (none with Grade 4), including single cases each of nausea, constipation, poor general condition, and an enzyme elevation. No cardiac toxicity was reported. Final evaluation of the ovarian cancer study revealed six patients with PR based on tumor lesions, plus two responders with tumor marker response including one case with normalization, for an overall response rate of 19% (one unconfirmed CR and seven partial responses). Median TTP and OS were evaluated as three and twelve months, respectively.

On September 14, 2011, positive final Phase 2 efficacy and safety data for zoptarelin doxorubicin in advanced endometrial cancer were presented at the European Society of Gynecological Oncology in Milan, Italy. The data showed that zoptarelin doxorubicin, administered as a single agent at a dosage of 267 mg/m^2 every three weeks was active, well tolerated and that OS was similar to that reported for modern triple combination chemotherapy, but was achieved with lower toxicity. The primary endpoint was the response rate as defined by the RECIST. Secondary endpoints included safety, TTP and OS.

In all, of 43 patients treated with zoptarelin doxorubicin, 39 were evaluable for efficacy. Efficacy confirmed by independent response review included two CR, ten PR, and 17 patients with stable disease ("SD"). Based on those data, the estimated overall response rate ("ORR") (ORR = CR+PR) was 30.8% and the CBR (CBR = CR+PR+SD) was 74.4%. Responses in patients previously treated with chemotherapy included one CR, one PR and two SDs in eight of the patients with prior use of platinum/taxane regimens. Median TTP and OS were seven months and 13.7 months, respectively. A final evaluation, not excluding non-evaluable cases, revealed the following results: two CR, eleven PR (including three patients with PR not confirmed at subsequent time point), and 17 patients with SD, for an ORR of 30.2% and CBR of 70%; median TTP and OS at seven and 15 months, respectively.

Overall, tolerability of zoptarelin doxorubicin was good and commonly allowed retreatment as scheduled. Severe (Grade 3 or 4) toxicity was mainly restricted to rapidly reversible leukopenia and neutropenia, associated with fever in only one patient who had been treated only three weeks after a surgery. Good tolerability of zoptarelin doxorubicin was also reflected by a low rate of severe non- hematological and possibly drug-related adverse events which included single cases each of nausea, diarrhea, fatigue, general health deterioration, creatinine elevation, and blood potassium decrease. No cardiac toxicity was reported.

On December 28, 2012, we announced that we had reached an agreement with the FDA with respect to a SPA for the ZoptEC Phase 3 registration trial of zoptarelin doxorubicin in endometrial cancer. The SPA agreement states that the proposed trial protocol design, clinical endpoints and planned analyses are acceptable to the FDA to support a regulatory submission. Final marketing approval depends on the results of efficacy, the adverse event profile and an evaluation of the benefit/risk of treatment demonstrated in the ZoptEC Phase 3 trial. This Phase 3 ZoptEC trial in women with locally advanced, recurrent or metastatic endometrial cancer who have progressed and who have received

one chemotherapeutic regimen with platinum and taxane (either as adjuvant first-line treatment), is an open-label, randomized, multicenter trial conducted in North America, Europe and Israel. The trial compares zoptarelin doxorubicin with doxorubicin as second line therapy and will involve approximately 500 patients. The primary efficacy endpoint of the ZoptEC trial is improvement in median Overall Survival.

On April 10, 2013, we announced the signing of a co-development and profit-sharing agreement with Ergomed for zoptarelin doxorubicin in endometrial cancer. Ergomed was selected as the contract clinical development organization to conduct the ZoptEC Phase 3 trial. Under the terms of the agreement, Ergomed has agreed to assume 30% (up to $10 million) of the clinical and regulatory costs for the trial which are estimated at approximately $30 million over the course of the study. Ergomed will

receive its return on investment based on an agreed single digit percentage of any net income received by Aeterna Zentaris for zoptarelin doxorubicin in this indication, up to a specified maximum amount.

On July 31, 2013, we announced that the first patient had been recruited and dosed for the ZoptEC Phase 3 trial in endometrial cancer.

On February 4, 2014, we announced that an article on the Phase 2 results for zoptarelin doxorubicin in endometrial cancer had been published in the February issue of the International journal of Gynecological Cancer. The results published in this article refer to the final evaluation of the Phase 2 trial in endometrial cancer described above.

Competitors for zoptarelin doxorubicin in Endometrial Cancer

At present, the Company is not aware of any approved drug product for the treatment of advanced and recurrent metastatic endometrial cancer in either the United States or Europe. There is also no systemic therapy approved in either the United States or Europe (except Germany) for treating advanced or recurrent endometrial cancer.

The following products are among some of the many products currently in clinical trial in endometrial cancer:

Product / mode of action*	Company*	Development Status*
Ixabepilone / microtubule inhibitor	Bristol-Myers Squibb	Phase 3
Letrozole / non-steroidal aromatase inhibitor	Novartis	Phase 2 and Phase 3
SAR245408 (XL-147)/PI3K inhibitor	Sanofi	Phase 2
BKM120/PI3K inhibitor	Novartis	Phase 1/2
TK1258/FGFR inhibitor	Novartis	Phase 1/2
GDC/0980 PI3K/mTOR inhibitor	Genentech	Phase 2
Lenvatinib (E7080)/ Multi-kinase inhibitor	Eisai	Phase 2
Sunitinib malate/Tyrosine kinase inhibitor	NCI	Phase 2

* Source: Competitor company's website and www.clinicaltrials.gov.

See also the risk factor entitled "Competition in our targeted markets is intense, and development by other companies could render our products or technologies non-competitive" in Item 3D of this annual report on Form 20-F.

Market Data - Endometrial Cancer

According to the American Cancer Society, endometrial cancer is the most common invasive gynecologic cancer in women in the United States, with an estimated 52,630 new cases expected to occur in 2014. This disease primarily affects postmenopausal women at an average age of 60 years at diagnosis. In the United States, it is estimated that approximately 8,590 women will die of endometrial cancer in 2014.

According to Datamonitor Healthcare (March 2010), a research and advisory firm that focuses on therapeutic, strategic and health market analysis and competitive intelligence, the incidence of endometrial cancer in the seven major pharmaceutical markets was 94,061 patients in 2010 and is forecasted to reach approximately 98,500 cases by 2019.

1.1.2 Zoptarelin doxorubicin – Triple-Negative Breast Cancer

On October 25, 2011, we announced that the FDA had granted Alberto J. Montero M.D. of the Sylvester Comprehensive Cancer Center, an IND approval for the initiation of a randomized Phase 2 trial in chemotherapy refractory triple-negative (ER/PR/HER2-negative) LHRH receptor-positive metastatic breast cancer with zoptarelin doxorubicin. Subsequently, the study was converted into a Company-sponsored study and is now conducted under our IND.

On February 20 2013, we announced that a first patient had been treated for the randomized Phase 2 trial in chemotherapy refractory triple-negative ("ER/PR/HER2-negative") luteinizing hormone-releasing hormone receptor ("LHRH-R")-positive metastatic breast cancer, with zoptarelin doxorubicin. Alberto J. Montero, MD, Assistant Professor, Department of Medicine, Division of Hematology/Oncology, Sylvester Comprehensive Cancer Center at the University of Miami Miller School of Medicine, is the lead investigator of this trial which also include sites at the

Universities of Regensburg and Goettingen, in Germany.

This is an open-label, randomized, two-arm, multicenter Phase 2 study which will involve up to 74 patients. Patients will be randomized in a 1:1 ratio into one of the two treatment arms: [Arm A] zoptarelin doxorubicin (267 mg/m^2 every 21 days) or [Arm B] SSC standard single agent cytotoxic chemotherapy at the discretion of the treating oncologist.

The primary study endpoint is median time of progression-free survival. Secondary endpoints include overall response rate, and overall survival. The study will also evaluate zoptarelin doxorubicin's toxicity profile and patients' quality of life relative to conventional cytotoxic chemotherapy.

On June 3, 2013, Stefan Buchholz, MD. at the Medical Center University of Regensburg, Germany, presented at the ASCO Annual Meeting the study design of the Phase 2 trial of zoptarelin doxorubicin in chemotherapy refractory triple negative LHRH-R positive metastatic breast cancer. The poster (abstract #TPS11124) was entitled "A randomized, Phase 2 trial of AEZS-108 in chemotherapy refractory triple negative (ER/PR/HER2-negative) LHRH-R positive metastatic breast cancer".

As part of our ongoing review to ensure optimization of our resources, we have decided to terminate this Phase 2 trial in triple-negative breast cancer.

1.1.3 Zoptarelin doxorubicin – Bladder Cancer

On May 12, 2010, we announced that the FDA had approved our IND application for zoptarelin doxorubicin in LHRH receptor-positive urothelial (bladder) cancer. Following this approval from the FDA, this trial will be conducted by Dr. Gustavo Fernandez at the Sylvester Comprehensive Cancer Center at the University of Miami's Miller School of Medicine, and will include up to 64 patients, male and female, with advanced LHRH receptor-positive urothelial (bladder) cancer. The study will be conducted in two parts: first, a dose-finding part in up to twelve patients; subsequently, the selected dose will be studied for its effect on PFS.

On December 14, 2010, we announced the initiation of the Phase 1/2 trial.

On July 26, 2012, we announced that preclinical data on zoptarelin doxorubicin in urinary bladder cancer were published in the online edition of Oncotarget. The article underlined that zoptarelin doxorubicin powerfully inhibited growth of bladder cancers in nude mice, exerted greater effects and was less toxic than doxorubicin ("DOX"). In contrast to DOX alone, which activated strong multidrug resistance mechanisms in RT-4 and HT-1197 cancers, zoptarelin doxorubicin had no or fewer such effects. Polymerase Chain Reaction ("PCR") assays and in vitro studies revealed differences in the action of zoptarelin doxorubicin and DOX on the expression of genes involved in apoptosis.

As part of our ongoing review to ensure optimization of our resources, we have decided to terminate this Phase 1/2 trial in bladder cancer.

1.1.4 Zoptarelin doxorubicin – Prostate Cancer

On August 5, 2010, we announced that the The National Institutes of Health ("NIH") had awarded Dr. Jacek Pinski, Associate Professor of Medicine at the Norris Comprehensive Cancer Center of the University of Southern California, a grant of $1.6 million over three years to conduct a Phase 1/2 study in refractory prostate cancer with zoptarelin doxorubicin. The study, entitled A Phase I/II Trial of AN-152 [AEZS-108] in Castration- and Taxane-Resistant Prostate Cancer, will enroll up to 55 patients and will be conducted in two portions: an abbreviated dose-escalation followed by a single arm, Simon Optimum two-stage design Phase 2 study using the dose selected in the Phase 1 portion. The primary objective of the Phase 2 portion is to evaluate the clinical benefit of zoptarelin doxorubicin in men with castration- and taxane-resistant metastatic prostate cancer, for which the presence of LHRH receptors has been confirmed.

On December 14, 2010, we announced the initiation of the investigator initiated Phase 1/2 trial.

On September 26, 2011, we announced positive interim data for the Phase 1 portion of the Phase 1/2 trial with zoptarelin doxorubicin in castration-and taxane-resistant prostate cancer at the European Society for Medical Oncology ("ESMO) meeting, Stockholm, Sweden. This is a single arm study with a Phase 1 lead-in to a Phase 2 clinical trial. The primary endpoint of the Phase 1 portion is safety. The primary objective of the Phase 2 portion is to evaluate the clinical benefit of zoptarelin doxorubicin for these patients. Twelve patients entered the study: three patients each received zoptarelin doxorubicin at the lower dose levels of 160 and 210 mg/m^2, and six patients at 267 mg/m^2. Data on ten patients were presented as two patients were too early for evaluation. Zoptarelin doxorubicin was

generally well tolerated and there were no dose limiting toxicities so far. The only Grade 3 and 4 toxicities were hematologic in nature. At the time, there were three Grade 4 toxicities (two at 210 mg/m^2 and one at 267 mg/m^2), all of which were asymptomatic. There were six Grade 3 toxicities including two cases of Grade 3 anemia after repeated courses (cycles five and six) and one case of febrile neutropenia that occurred during cycle one. Signs of therapeutic activity included five patients with Prostate Specific Antigen ("PSA") regression. One of these patients treated at the lowest dose level, received eight treatment cycles because the patient demonstrated continued clinical benefit. Three out of

four evaluable patients with radiologic evaluable disease achieved stable disease per RECIST. The Phase 2 extension is planned after completion of the toxicity assessment in the final dose level of the Phase 1 portion of the study. In correlative studies, drug uptake was demonstrated for the first time in captured circulating tumor cells of patients, thus validating the principle of targeted tumor therapy with zoptarelin doxorubicin in a clinical setting.

On February 3, 2012, we reported updated results for the Phase 1 portion of the ongoing Phase 1/2 study of zoptarelin doxorubicin in prostate cancer.

The results were based on 13 patients who had been previously treated with androgen-deprivation therapy (LHRH agonist) and at least one taxane-based chemotheraphy regimen, who were treated on three dose levels of zoptarelin doxorubicin: three at 160 mg/m^2, three at 210 mg/m^2, and seven at 267 mg/m^2. Overall, zoptarelin doxorubicin was well tolerated among this group of heavily pretreated older patients. There were two dose-limiting toxicities, each of which having been a case of asymptomatic Grade 4 neutropenia at the 267 mg/m^2 dose level and both patients fully recovered. The Grade 3 and 4 toxicities were primarily hematologic. There was minimal non-hematologic toxicity, most frequently fatigue and alopecia.

Despite the low doses of zoptarelin doxorubicin in the first cohorts, there was some evidence of antitumor activity. One patient received eight cycles (at 210 mg/m^2) due to continued benefit. Among the five evaluable patients with measurable disease, four achieved stable disease. At the time of submission of the abstract, a decrease in PSA was noted in six patients. Six of 13 (46%) treated patients received at least five cycles of therapy with no evidence of disease progression at twelve weeks. Correlative studies on circulating tumor cells ("CTC") demonstrated the uptake of zoptarelin doxorubicin into the targeted tumor.

On November 12, 2012, we announced the initiation of the Phase 2 portion of the ongoing Phase 1/2 study of zoptarelin doxorubicin in prostate cancer. The primary endpoint of the Phase 2 portion is to evaluate the clinical benefit of zoptarelin doxorubicin for these patients. Secondary endpoints include toxicity, time to RECIST and PSA progression, RECIST response rate for patients with measurable disease, PSA response rate, pain palliation and overall survival.

On June 3, 2013, we announced that final data for the Phase1 portion of the ongoing Phase 1/2 trial with zoptarelin doxorubicin in prostate cancer, demonstrated the compound's promising anti-tumor activity. Results were presented by lead investigator, Jacek Pinski, MD, PhD, of the USC Norris Comprehensive Cancer Center, during a poster session at the ASCO Annual Meeting in Chicago.

Eighteen men with a median of two prior chemotherapy regimens (range 1/5) and a median PSA of 106.4 ng/mL (range 8.4-1624.0) were enrolled. The dose of zoptarelin doxorubicin was escalated from 160 mg/m^2 to 210 mg/m^2 then to 267 mg/m^2. There were two Dose-Limiting Toxicities ("DLT") in the seven patients receiving zoptarelin doxorubicin at a dose of 267 mg/m^2 (grade 4 neutropenia), establishing 210 mg/m^2 as the Maximum Tolerated Dose ("MTD"). Significant non-hematologic toxicities included one case of grade 3 nausea. No cardiotoxicity was seen on serial evaluation and six patients completed six cycles. Internalization of zoptarelin doxorubicin was consistently visualized in CTCs 1 to 3 hours after dosing. Maximal PSA response was stable or decreased in 8 of 18 men. Among the 15 evaluable patients with measurable disease, ten achieved stable disease and a drop in PSA was noted in three patients. The MTD of zoptarelin doxorubicin in this indication is 210 mg/m^2, which is below the MTD reported in women with refractory endometrial and ovarian cancer.

The Phase 2 portion of that Phase 1/2 trial is ongoing.

1.1.5 AEZS-137 (Disorazol Z) / AEZS-138 (LHRH-Disorazol Z)

In search of new antitumor agents, we found that disorazol Z (AEZS-137), isolated from the myxobacterium Sorangium cellulosum, possess cytotoxicity in the picomolar range in a panel of different tumor cell lines. Inhibition of tubulin polymerization, cell cycle arrest and efficient induction of apoptosis, have been identified as modes of action.

On March 24, 2011, we were awarded a $1.5 million grant from the German Ministry of Education and Research to develop, up to the clinical stage, cytotoxic conjugates of the proprietary cytotoxic compound AEZS-137 and peptides targeting G-protein coupled receptors, including the LHRH receptors. The compounds being developed will combine the targeting principle successfully employed in Phase 3 with zoptarelin doxorubicin with the novel cytotoxic disorazol Z. Furthermore, diagnostic tools systematically assessing the receptor expression in tumor specimens will be

developed to allow the future selection of patients and tumor types with the highest chance of benefiting from this personalized medicine approach. The grant was payable as a partial reimbursement of qualifying expenditures over a three-year period, until January 31, 2014. The qualified project was performed with Morphisto GmbH and the Helmholtz Institute in Saarbrücken, Germany, which received additional funding of approximately US$0.7 million. Researchers from the departments of Gynecology and Obstetrics at both the University of Göttingen and the University of Würzburg, Germany, were also part of the collaboration.

On November 16, 2011, we announced the presentation of a poster at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics on encouraging preclinical data for AEZS-137. The data showed that AEZS-137 possesses cytotoxicity in a highly diverse panel of 60 different tumor cell lines, and also underlined the identification of important aspects of this novel natural compound's mechanism of action. AEZS-137 has been identified as a tubulin binding agent with highly potent antitumor properties. Cell cycle analysis revealed that AEZS-137 arrested cells in the G2/M cell cycle phase and subsequently induced apoptosis with remarkable potency, as shown by sub-nanomolar EC50 values. Currently, experiments are under way to determine the tubulin binding site for disorazol Z and to identify further mechanisms of action of this novel highly potent agent. To expand our zoptarelin doxorubicin technology platform, we aim to evaluate the utility of disorazol Z as a cytotoxic component in a drug-targeting approach utilizing GPCR ligands as the targeting moieties for the treatment of GPCR over-expressing cancers.

On April 10, 2013, we announced at the American Association for Cancer Research's ("AACR") encouraging updated proof-of-concept results for Disorazol Z cytotoxic conjugates, such as AEZS-125 and AEZS-138, in human ovarian and endometrial cancer xenograft models. Data demonstrated that conjugates of D-Lys6-LHRH and Disorazol Z retained strong binding to the LHRH receptor and showed potent inhibition of tubulin polymerization. Cellular cytotoxicity of the conjugates was in the low nanomolar EC50 range. Increased cytotoxicity in cells over-expressing the LHRH receptor, support receptor targeting as a mechanism of action. The LHRH receptor-dependent efficacies of Disorazol Z - D-Lys6-LHRH conjugates in vitro and in mouse xenograft models that were presented, support the principle of tumor targeting by the LHRH receptor as already employed by the drug candidate zoptarelin doxorubicin, which is currently in a ZoptEC Phase 3 study in endometrial cancer and in a Phase 2 study in prostate cancer.

On February 11, 2014, at the 11th International Symposium on GnRH, in Salzburg, Austria, we presented further data on the mechanism of action and proof of concept of the disorazol Z cytotoxic conjugate, AEZS-138, which had led to the initiation of its preclinical development during the second quarter of 2013.

As part of our ongoing review to ensure the optimization of our resources, we are currently evaluating our options for this project.

1.2 TUBULIN INHIBITORS / VASCULAR TARGETING AGENTS

1.2.1 AEZS-112

Tubulin is a protein found in all cells that plays an important role during cell division in that, it helps to transmit genetic information to the daughter cells. Inhibition of this process leads to the death of the affected cell. The antitumor agents taxol and vincristine, which are widely used in cancer therapy, are based on this principle. Both compounds are expensive natural substances and cause severe side effects when used in humans.

We are currently identifying and developing novel tubulin inhibitors which, compared with currently used products, exhibit improved efficacy in animal models, have a more acceptable side effect profile, an incomplete or no cross-resistance and are administered orally.

AEZS-112 is a drug development candidate with a favorable safety and tolerability profile showing excellent in vivo activity in various tumor models including mammary, colon, melanoma and leukemia cancers at acceptable and very well tolerated doses administered orally once weekly. This compound acts through three mechanisms of action. Strong anticancer activity is combined with proapoptotic and antiangiogenic properties. AEZS 112 inhibits the polymerization of tubulin, destroys the mitotic spindle of the cancer cells and inhibits topoisomerase II activity. AEZS-112 arrests the cancer cells in the G2M cell cycle phase at a nanomolar concentration and induces apoptosis. AEZS-112 is not cross-resistant to cisplatin, vincristine and doxorubicin in cell lines resistant to these drugs.

On January 8, 2007, we announced the initiation of a Phase 1 trial for AEZS-112 in patients with solid tumors and lymphoma. This open-label, dose-escalation, multicenter, intermittent treatment Phase 1 trial was conducted in the United States with Daniel D. Von Hoff, M.D., Senior Investigator at the Translational Genomics Research Institute in Phoenix, AZ, as the lead investigator. The trial included up to 50 patients with advanced solid tumors and lymphoma who have either failed standard therapy or for whom no standard therapy exists. Patients received a once-a-week oral administration of AEZS-112 for three consecutive weeks, followed by a one-week period without treatment. The cycles were repeated every four weeks based on tolerability and response, basically planned for up to four cycles, but allowing for continuation in case of potential benefit for the patient. The starting dose of AEZS-112 in this study was

13 mg/week, with doubling of doses in subsequent cohorts in the absence of significant toxicity. The primary endpoint of the Phase 1 trial focused on determining the safety and tolerability of AEZS-112 as well as establishing the recommended Phase 2 dose and regimen. Secondary endpoints were aimed at establishing the pharmacokinetics and determining the efficacy based on standard response criteria.

Results of this Phase 1 study were presented in April 2009 at the AACR meeting. In part I, 22 patients (twelve men / ten women) were studied on seven dose levels ranging from 13 to 800 mg/week. In all, 62 treatment cycles were administered. In part II, the weekly dose was split into three doses taken eight hours apart. Ultimately, 22 patients (twelve men / ten women) were studied on five dose levels ranging from 120 to 600 (= 200 x 3) mg/week. As at April 1, 2009, 62 treatment cycles had been administered (mean 3.2/patient) and treatment had been ongoing in eight patients. SD for more than twelve weeks was observed in 16 patients; four more patients were ongoing at less than twelve weeks. Prolonged courses of SD ranging from 20 to 35+ weeks were observed in nine patients with the following primary cancer types: trachea (39+), tongue (30+), thyroid (29+), prostate and melanoma (28), non-small cell lung cancer (26+), pancreas and 2x colorectal (20). Except for one patient with a background of gastrointestinal problems ("GI") who had dose-limiting GI reactions and electrolyte loss at a dose of 200 x 3 mg/week, no clinically relevant drug-related adverse events or changes in laboratory parameters were observed. AEZS-112 was shown to be metabolically stable in human plasma. As predicted by pharmacokinetic modelling based on data from part I of the study, the split-dose scheme led to a higher Cmax and trough values after administration of comparable doses. Those preliminary results showed that a maximum tolerated dose for weekly dosing has not been defined so far. However, prolonged courses of stable disease in both parts of the study were an encouraging observation.

Completion of this Phase 1 trial was announced on September 21, 2009. Stable disease with time to failure ranging from 20 to 60+ weeks was achieved in twelve patients with various cancer types, including melanoma and cancers of the colon/rectum, lung, pancreas, prostate, tongue, trachea and thyroid. In several of these patients, the duration of stabilization exceeded the duration of disease control on previous treatment regimens. Except for a dose-limiting gastrointestinal reaction in a patient with pre-existing GI problems, no clinically relevant drug-related adverse events or changes in laboratory safety parameters were observed.

In 2011, we developed a higher concentration oral formulation of AEZS-112 in order to improve patient compliance. As part of our ongoing review to ensure the optimization of our resources and as the compound did not reach the expected outcome in terms of formulation, we are currently in the process of evaluating our options for this compound.

1.3 IMMUNOTHERAPY / VACCINES

1.3.1 AEZS-120

AEZS-120 is a preclinical tumor vaccine. The general principle of active tumor vaccines is the induction of a cellular and/or humoral immune response which is capable of attacking the tumor. AEZS-120 is a live recombinant oral tumor vaccine candidate based on Salmonella typhi Ty21a as a carrier strain. Salmonella typhi Ty21a is an approved oral typhoid vaccine which has been safely applied in more than 250 million doses. The molecular basis of AEZS-120 is the recombinant expression of the fusion protein between cholera toxin B (CtxB) and prostate specific antigen ("PSA"), and the recombinant expression of two components of the hemolysin secretion system (HlyB and HlyD) as well as the signal component HlyA which allow the secretion of the fusion protein by the attenuated approved carrier strain S. typhi Ty21a.

The relevant features with respect to activity as a tumor vaccine can be divided into two parts: A) adjuvant elements for optimal induction of innate and adaptive immunity; and B) the tumor antigen itself.

In the case of AEZS-120, the tumor antigen is PSA which is expressed in the majority of prostate cancer cases and is employed as a tumor antigen in several prostate cancer vaccines in development. Therefore, PSA can be considered as a valid antigen for prostate cancer vaccines.

The adjuvant activity is provided by two elements: the live bacterial carrier itself and the fusion to CtxB.

An important property of AEZS-120 is the oral application mode, which is based on the carrier S. typhi Ty21a. This strain is approved as a vaccine against typhoid fever and has preserved some features of virulent S. typhi strains which are relevant for the use of S. typhi Ty21a as a vaccine carrier. Virulent S. typhi is a pathogen which leads to systemic infection after oral uptake. Several virulence factors allow the survival within the gastro-intestinal tract and the crossing of the intestinal barrier. These features are, at least in part, also intact in the attenuated live vaccine S. typhi Ty21a allowing oral application with retained immunogenicity.

However, in particular, the cellular immune response against recombinantly expressed antigens, which is important for anti-tumor immunity, has been described as being suboptimal if the antigen is expressed within the carrier cell. A substantial enhancement can be achieved via secretion of the recombinant antigen. In gram negative bacteria, like Salmonellae, protein secretion requires the activity of protein secretion machineries. Several types of secretion systems with different levels of complexity have been described. The principle of AEZS-120 is based on the recombinant expression of prostate-specific antigen fused to the B subunit of cholera toxin and a secretion signal in the presence of the Escherichia coli type I hemolysin secretion system. The proprietary system allows the secretion of the antigen together with an immunological adjuvant which has been demonstrated to be required for optimal induction of CD8 T-cell responses by recombinant Salmonella based bacterial vaccines. The proof-of-concept was already demonstrated for the mouse homologue of AEZS-120 in a mouse tumor challenge model and is supported by several patent applications filed in 2007 and 2009.

In 2007, AEZS-120 was selected by the Company as its first preclinical development candidate of an antitumor vaccine.

On July 20, 2011, we reached a key milestone in this non-clinical development program of AEZS-120, which encompassed the full development of a GMP process, including GMP production and quality testing of a clinical batch, as well as a non-clinical safety and toxicology package. AEZS-120 has been developed through a research collaboration with the Department of Medical Radiation Biology and Cell Research, and the Department of Microbiology of the University of Würzburg, Germany. The collaboration was funded with a total of $890,000 for us and $870,000 for the university partner by the German Ministry of Education and Research (BMBF) for a period of three years. In accordance with this grant, 50% of our preclinical development costs and 100% of those of our university partner were reimbursed by the German Ministry of Science and Education. In addition, as part of the collaboration, a melanoma vaccine based on the recombinant expression of a modified B-Raf protein has been generated.

On October 2, 2012, we announced the presentation of a poster on AEZS-120 during the 32nd Congress of the Société Internationale d'Urologie in Fukuoka, Japan. The poster, entitled "Preclinical Proof of Concept and Characterization of AEZS-120, a Therapeutic Oral Prostate Cancer Vaccine Candidate Based on Live Recombinant Attenuated Salmonella", underlined the feasibility of an oral therapeutic vaccination approach against prostate cancer. The production, release, pharmacology, safety and toxicology program was conducted in agreement with the regulatory authorities and successfully finalized. The conclusions were:

- The proof-of-concept has been shown in a tumor-challenge mouse model using the anticipated clinical application schedule.
- Biosafety and biodistribution studies did not reveal a different safety profile compared to the carrier strain.
- Pharmacological and toxicological studies did not reveal differences to the approved carrier strain.
- In all, the non-clinical studies suggest that the safety and toxicological profile of AEZS-120 is similar to the approved carrier strain S. typhi Ty21a, which has already been safely administered in more than 250 million doses.

GMP material for clinical use has been produced and released, and we have approval from the Danish regulatory authorities as well as the ethics committee for the initiation of a proof-of-concept Phase 1 trial in prostate cancer. However, as part of our ongoing review to ensure the optimization of our resources, we are currently evaluating our options for this project.

1.4 SIGNAL TRANSDUCTION INHIBITORS

1.4.1 Erk/PI3K inhibitors and dual kinase inhibitors

The Ras/Raf/Mek/Erk and the PI3K/Akt signaling pathways are prime targets for drug discovery in proliferative diseases such as cancer. The results of research to date indicate that both the MAPK and the PI3K signaling pathways

represent therapeutic intervention points for the clinical treatment of malignant tumors.
Our multi-parameter optimization program for kinase inhibitor selectivity, cellular efficacy, physicochemical and in vitro ADMET properties has led to the identification of small molecular compounds with a unique kinase selectivity profile. Our kinase research program comprises the investigation of different compounds for single Erk inhibition, single PI3K inhibition and dual Erk/PI3K kinase inhibition.

1.4.1.1 AEZS-129

On November 17, 2010, we presented a poster on encouraging preclinical results for AEZS-129, a novel orally active compound with antitumor effects, at the 22nd EORTC-NCI-AACR Symposium on Molecular Targets and Cancer Therapeutics in Berlin, Germany. AEZS-129 has been identified as a highly potent and selective pan-PI3K inhibitor. The compound inhibits the PI3K/Akt signaling pathway both in vitro and in vivo and leads to growth inhibition of tumor cells. The compound was well tolerated during the four-week treatment period and showed substantial tumor growth inhibition in different mouse xenograft tumor models.

On March 22, 2011, we presented preclinical results for AEZS-129 at the Informa Life Sciences Protein Kinases Congress in Berlin, Germany. AEZS-129 was identified as a potent inhibitor of class I PI3Ks lacking activity against mTOR. Lack of mTOR activity is considered to potentially lead to a better safety profile. In biochemical and cellular assays, AEZS-129 demonstrated favorable properties in early in vitro ADMET screening, including microsomal stability, plasma stability and screening against a safety profile composed of receptors, enzymes and cardiac ion-channels. In vitro, the compound was shown to be a selective ATP-competitive inhibitor of PI3K with a broad antiproliferative activity against a broad panel of tumor cell lines. In vivo, AEZS-129 showed excellent plasma exposure and significant tumor growth inhibition in several tumor xenografts models, including A-549 (lung), HCT-116 (colon) and Hec1B (endometrium). These data suggest that AEZS-129 is a promising compound for clinical intervention of the PI3K/Akt pathway in human tumors.

1.4.1.2 AEZS-136

On April 3, 2012, we announced that a poster on AEZS-136 showed the compound's unique inhibition and promising activity against PI3K and Erk signaling pathways, as well as being well tolerated. The poster, entitled "Dual inhibition of PI3K and Erk1/2 shows synergy and efficacy in human tumor cells, either by using drug combinations or novel dual PI3K/Erk inhibitors", was presented at the AACR Annual Meeting in Chicago.

The conclusions were as follows:

- Effective dual targeting of Raf-Mek-Erk and PI3K-Akt pathway.
- Unique inhibitor with excellent activity against PI3K and Erk.
- Induction of cell cycle arrest in G1 phase and apoptosis.
- Broad anti-proliferative activity in vitro.
- Favorable in vitro ADMET and in vivo PK profile.
- Well tolerated up to daily doses of 90mg/kg for 4 weeks.
- In vivo antitumor efficacy after oral administration.

On August 13, 2012, we announced the presentation of a poster on AEZS-136 during the 244th National Meeting of the American Chemistry Society in Philadelphia. The data outlined the compound's unique inhibition and excellent preclinical activity against PI3K and Erk signaling pathways, as well as being well tolerated. AEZS-136 is an integral part of our kinase research program comprising the investigation of different compounds for single Erk inhibition, single PI3K inhibition and dual Erk/PI3K kinase inhibition. AEZS-136 selectively inhibits the kinase activity of Erk 1/2 and class 1 PI3Ks, enabling simultaneous inhibition of the Raf-Mek-Erk and the PI3K-Akt signaling cascades. AEZS-136 was discovered using our proprietary compound library and high throughput screening technology.

As part of our ongoing review to ensure the optimization of our resources, we are currently evaluating the next steps for our Erk/PI3K inhibitors program.

1.4.2 Perifosine

On March 11, 2013, we announced that the Phase 3 trial in multiple myeloma was discontinued after an interim analysis by an independent Data Safety Monitoring Board reported that it was highly unlikely the study would achieve a significant difference in its primary endpoint of progression-free survival. We therefore decided not to make any further investment in the development of perifosine.

Perifosine remains partnered with Yakult in Japan, Handok in Korea and Hikma in the MENA region for various cancer indications.

In addition, perifosine remains the object of certain investigator-initiated studies in different indications such as neuroblastoma, glioma, pediatric solid tumors and other indications.

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2.0 ENDOCRINOLOGY

2.1 MACIMORELIN

Macimorelin, a ghrelin agonist, is a novel orally active small molecule that stimulates the secretion of growth hormone by binding to the ghrelin receptor (GHSR-1a). It has potential uses in both endocrinology and in oncology indications.

In endocrinology, the FDA has accepted for substantive review our NDA for MACRILEN™ for the evaluation of AGHD. MACRILEN™ is a peptidomimetic ghrelin receptor agonist with growth hormone secretagogue activity. If approved, MACRILEN™ will be the first orally administrated drug indicated for the evaluation of AHGD by evaluating the pituitary gland secretion of growth hormone in response to an oral dose of the product. MACRILEN™ has been granted orphan-drug designation by the FDA for use in evaluating growth hormone deficiency ("GHD"). We own the worldwide rights to MACRILEN™.

In oncology, an IND has been granted for a Phase 2A trial with macimorelin in cancer-induced cachexia, a disease which leads to significant weight loss and diminished functional performance. Since ghrelin agonists such as macimorelin have been shown to stimulate food intake and increase body weight in rats and mice, macimorelin could lead to better quality of life for patients with cancer-induced cachexia. Ghrelin agonists have been in clinical trials for over a decade and have generally demonstrated good safety and efficacy profiles.

2.1.1 MACRILEN™ (macimorelin) – Use for evaluation of AGHD

On October 19, 2009, we announced that we had initiated activities intended to complete the clinical development of MACRILEN™ for use in evaluating growth hormone deficiency. We had already assumed the sponsorship of the IND and discussed with the FDA the best way to complete the ongoing Phase 3 clinical trial and subsequently file an NDA for approval of MACRILEN™ for use in evaluating AGHD.

The pivotal Phase 3 trial was designed to investigate the safety and efficacy of the oral administration of MACRILEN™ as a growth hormone stimulator for use in evaluating AGHD. It was accepted by the FDA that for the ongoing part of the study, MACRILEN™ would not be tested against a comparator drug, as Geref® had been removed from the market.

On June 21, 2010, we presented positive data at the 92nd ENDO Meeting on MACRILEN™ for evaluation and therapeutic use. The preclinical data showed that MACRILEN™ is a potent and safe oral synthetic GH-releasing compound with potential utility in evaluating growth hormone deficiencies.

On July 14, 2010, we announced the presentation of a poster on MACRILEN™, entitled Use of the Orally Active Ghrelin Mimetic AEZS-130 as a Simple Test for the Diagnosis of Growth Hormone (GH) Deficiency (GHD) in adults (AGHD). Merriam G.R., Yuen K., Bonert V., Dobs A, Garcia J., Kipnes M., Molitch M., Swerdloff R., Wang C., Cook D., Altemose I. and Biller B. This poster was presented at the Seventh International Congress of Neuroendocrinology, in Rouen, France.

On October 5, 2010, at the Fifth International Congress of the Growth Hormone Research Society and the Insulin-like Growth Factors Society, we announced that, after the interim Phase 3 analysis, MACRILEN™ demonstrated the potential to provide a simple, well tolerated and safe oral product for use in evaluating AGHD.

On December 20, 2010, we announced we had reached agreement with the FDA on a SPA for MACRILEN™, enabling the Company to complete the ongoing registration study required to gain approval for use in evaluating AGHD.

The first part of the study, conducted by our former partner, Ardana, was a two-way cross-over study and included 42 patients with confirmed AGHD or multiple pituitary hormone deficiencies and a low insulin-like growth factor-I. A control group of 10 subjects without AGHD were matched to patients for age, gender, body mass index and (for females) estrogen status.

On July 26, 2011, we announced the completion of the Phase 3 study of MACRILEN™ as a first oral product for use in evaluating AGHD and the decision to meet with the FDA for the future filing of an NDA for the registration of MACRILEN™ in the United States.

On August 30, 2011, we announced favorable top-line results of our completed Phase 3 study with MACRILEN™ as a first oral product for use in evaluating AGHD. The results showed that MACRILEN™ had reached its primary endpoint demonstrating >90% area-under-the-curve ("AUC") of the Receiver Operating Characteristic ("ROC") curve, which determines the level of specificity and sensitivity of the product. Importantly, the primary efficacy parameters show that the study achieved both specificity and sensitivity at a level of 90% or greater. In addition, eight of the ten newly

enrolled AGHD patients were correctly classified by a pre-specified peak GH threshold level. The use of MACRILEN™ was shown to be safe and well tolerated overall throughout the completion of this trial.

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On June 26, 2012, we announced that the final results from a Phase 3 trial for MACRILEN™ showed that the drug is safe and effective in evaluating AGHD. Jose M. Garcia, MD, PhD, of the Baylor College of Medicine and the Michael E. DeBakey VA Medical Center, disclosed these data during an oral presentation at the 94th ENDO Annual Meeting and Expo in Houston. The study had originally been designed as a cross-over trial of MACRILEN™ vs. growth hormone-releasing hormone (GHRH) + L-Arginine (ARG) in AGHD patients and in controls matched for body mass index ("BMI"), estrogen status, gender and age. After 43 AGHD patients and ten controls had been tested, GHRH became unavailable. The study was completed by testing ten more AGHD patients and 38 controls with MACRILEN™ alone. Of the 53 AGHD subjects enrolled, 52 received MACRILEN™, and 50 who had confirmed AGHD prior to study entry were included in this analysis, along with 48 controls. Two AGHD subjects could not be matched due to the combination of young age, high BMI and estrogen use. The objective of this clinical trial was to determine the efficacy and safety of MACRILEN™ in the evaluating of AGHD. Mean peak growth hormone ("GH") levels in AGHD patients and controls following MACRILEN™ administration were 2.36ng/mL (range 0.03-33) and 17.71ng/mL (range 10.5-94), respectively. The ROC plot analysis yielded an optimal GH cut-point of 2.7ng/mL, with 82% sensitivity, 92% specificity and a 13% misclassification rate. Obesity (BMI>30) was present in 58% of cases and controls, and peak GH levels were inversely associated with BMI in controls. Adverse events ("AE") were seen in 37% of AGHD patients and in 21% of controls following MACRILEN™. In contrast, 61% of AGHD subjects and 30% of controls experienced AEs with L ARG+GHRH. The most common AEs after MACRILEN™ were unpleasant taste (19.2%) and diarrhea (3.8%) for the AGHD patients and unpleasant taste (4.2%) and diarrhea (4.2%) for the matched controls. No clinically meaningful changes from baseline in ECG results during the study for AGHD patients; however, one control subject had an ECG change (T wave abnormality and QTc interval prolongation) one hour after treatment with MACRILEN™ that was considered a serious treatment-related adverse event and resolved spontaneously within 24 hours. The subject had been pre-treated with citalopram, a drug that was later reported by the FDA to be associated with QT prolongation, although the patient had stopped this medication seven days prior to dosing. Overall, this study demonstrated that MACRILEN™ is safe and effective for use in evaluating AGHD.

On August 7, 2012, the United States Patent and Trademark Office granted us a patent for the use of MACRILEN™ as a product to be used in evaluating AGHD. Filed on February 19, 2007, the patent (US 8,192,719 B2), entitled "Methods and Kits to Diagnose Growth Hormone Deficiency by Oral Administration of EP1572 or EP1573 Compounds", became effective as of June 5, 2012 and will expire on October 12, 2027. The corresponding composition of matter patent (US 6,861,409 B2), filed on June 13, 2001 and granted on March 1, 2005, will expire on August 1, 2022, with the possibility of a patent term extension of up to five years.

On September 25, 2012, the European Patent Office granted us a patent for the use of MACRILEN™ related to methods and kits for use in relation to the evaluation of GHD in a human or animal subject. Filed on February 19, 2007, the patent, (EP #1 984 744 B1) entitled "Methods and Kits to Diagnose Growth Hormone Deficiency", was effective as of September 19, 2012 following its publication in the European Patent Bulletin, and it will expire on February 19, 2027.

On September 26, 2012, we received notification from the FDA that Fast Track designation previously applied for had not been granted for MACRILEN™ as a product for use in evaluating AGHD.

On October 18, 2012, we announced that results from a multicenter open-label Phase 3 trial for MACRILEN™ demonstrated that the drug is safe and effective in evaluating AGHD. George R. Merriam, MD, Director of the Clinical Study Unit at the Veterans Affairs Puget Sound Health Care System, and Professor of Medicine at the University of Washington, Seattle and Tacoma, WA, disclosed these data at the 6th International Congress of the GRS and IGF Society in Munich, Germany. His presentation confirmed data previously presented by Jose M. Garcia, MD, Ph.D., of the Baylor College of Medicine and the Michael E. DeBakey Veterans Affairs Medical Center, at the 94th ENDO Meeting in Houston, Texas in June 2012. Dr. Merriam's presentation drew attention to the effect of BMI on optimizing the cut-off values to improve the sensitivity and specificity of the test. Responses in normal subjects classified as obese, with BMI's above 30, were significantly lower than in leaner subjects. Since GH deficiency can lead to increased body fat, many of the patients also met criteria for obesity, and therefore, a lower peak GH cut-off is more accurate in separating obese normals from obese patients. Based upon these study results, a cut-off of 2.7 µg/L was optimal for subjects with a BMI≥30 and a cut-off of 6.8 µg/L for subjects with a BMI<30. Age had a weaker effect on test performance and gender made no difference. Thus GH stimulation with oral MACRILEN™ may provide a

simple, rapid, safe, and well-tolerated product used in evaluating AGHD, with accuracy comparable to that of the GHRH-ARG test.

On January 6, 2014, we announced that the FDA had accepted for substantive review our NDA for our orally available peptidomimetic ghrelin receptor agonist with growth hormone secretagogue activity, MACRILEN™, for the evaluation of AGHD. The acceptance for filing of the NDA indicates that the FDA has determined that the application is sufficiently complete to permit a substantive review. The NDA, submitted on November 5, 2013, seeks approval for the commercialization of MACRILEN™, which, if approved, will be the first orally administered drug indicated for the evaluation of AGHD by evaluating the pituitary gland secretion of growth hormone in response to an oral dose of the product. The application is subject

to a standard review by the FDA. MACRILEN™ benefits from patent protection covering major markets; in particular, the product is protected in the U.S. at least until October 2027. Phase 3 data have demonstrated the compound to be well tolerated, with accuracy comparable to available intravenous and intramuscular testing procedures.

Throughout the remainder of 2014, we expect to advance the pre-launch activities related to the initial commercialization of MACRILEN™ in AGHD in the U.S. market. As noted above, our NDA is currently under substantive review by the FDA. Subject to the successful review and acceptance of our NDA, we expect to make MACRILEN™ available by prescription in the U.S. as soon as commercially practicable following final regulatory approval.

We intend to build a commercial infrastructure necessary to access the physicians who perform the majority of AGHD tests (endocrinologists) along with the major centers of AGHD influence. Commercial initiatives are likely to include the targeted selection, hiring and deployment of a contracted sales force by the end of 2014. The targeted marketing efforts of our sales force will reach endocrinology specialists of AGHD. We believe these efforts will enable the realization of a substantial portion of the potential commercial opportunity for MACRILEN™.

Competitors for MACRILEN™ in the evaluation of AGHD

Competitors for MACRILEN™ as a product for the evaluation of AGHD are principally the diagnostic tests currently performed by endocrinologists, although none of these tests are approved by the FDA for this purpose.

The most commonly used diagnostics tests for GHD are:

- Measurement of blood levels of Insulin Growth Factor ("IGF")-1, which is typically used as the first test when GHD is suspected. However, this test is not used to definitively rule out GHD as many growth hormone deficient patients show normal IGF-1 levels;
- Insulin Tolerance Test ("ITT"), which is considered to be the "gold standard" for GH secretion provocative tests but requires constant patient monitoring while the test is administered and is contra-indicated in patients with seizure disorders, with cardiovascular disease and in brain injured patients and elderly patients. ITT is administered i.v.;
- GHRH + Arginine test, which is an easier test to perform in an office setting and has a good safety profile but is considered to be costly to administer compared to ITT and Glucagon. This test is contra-indicated in patients with renal failure. GHRH + Arginine is approved in the EU and has been proposed to be the best alternative to ITT, but it is no longer available in the United States. This test is administered i.v.; and
- Glucagon test, which is simple to perform and is considered relatively safe by endocrinologists but is contraindicated in malnourished patients and patients who have not eaten for more than 48 hours. Since there is a suspicion that this test may cause hypoglycemia, it may not be appropriate in diabetic populations. This test is administered i.m.

Oral administration of MACRILEN™ offers more convenience and simplicity over the current GHD tests used, requiring either i.v. or i.m. administration. Additionally, MACRILEN™ may demonstrate a more favorable safety profile than existing diagnostic tests, some of which may be inappropriate for certain patient populations e.g. diabetes mellitus or renal failure, and have demonstrated a variety of side effects which MACRILEN™ has not thus far. These factors may be limiting the use of GHD testing and may enable MACRILEN™ to become the product of choice in evaluating AGHD.

Market Data - AGHD

There are approximately 36,000 AGHD tests performed annually in the U.S. Based on published information from the U.S. Centers for Disease Control and Prevention, different scientific publications and by Navigant Research, we estimate that the total potential U.S. market for AGHD evaluation is approximately 158,000 tests per year, including the evaluation of patients who have suffered traumatic brain injury ("TBI"). In patients with TBI, a GHD is frequent and may contribute to cognitive sequel and reduction in quality of life. GHD develops in approximately 19% of both severe and moderate hospitalized TBI victims (scientific publications: Agha et al., British Journal of Neurosurgery, 2007, Fernandez-Rodrigues et al., Frontiers in Endocrinology, 2011 and Popovic et al., Frontiers of Hormone Research, Basel, Karger, 2005).

2.1.2 Macimorelin – Cancer Cachexia

On November 28, 2011, we announced that the FDA had granted Jose M. Garcia, M.D., Ph.D., Assistant Professor, Division of Diabetes Endocrinology and Metabolism, Departments of Medicine and Molecular and Cell Biology, Baylor College of Medicine and Michael E. DeBakey Veterans Affairs Medical Center, in Houston Texas, an IND

approval for the initiation of a Phase 2A trial to assess the safety and efficacy of repeated doses of macimorelin in patients with cancer cachexia. Cachexia,

which is characterized by diminished appetite and food intake in cancer patients, is defined as an involuntary weight loss of at least 5% of the pre-illness body weight over the previous 6 months.

On March 8, 2012, we announced that the Michael E. DeBakey Veterans Affairs Medical Center, in Houston, Texas, had initiated the Phase 2A trial assessing the safety and efficacy of repeated doses of macimorelin in patients with cancer cachexia. The study is conducted under a CRADA with the Michael E. DeBakey Veterans Affairs Medical Center, which is funding the study. This is a double-blind, randomized, placebo-controlled Phase 2A trial to test the effects of different doses of macimorelin in 18 to 26 patients with cancer cachexia. The study will involve three sequential groups receiving differing doses of macimorelin. Each dose group will have six patients who will receive macimorelin and two to four patients who will receive a placebo. The primary objective of the study is to evaluate the safety and efficacy of repeated oral administration of macimorelin at different doses daily for one week in view of developing a treatment for cachexia.

The study is ongoing with patient enrollment not yet completed.

2.2 LHRH ANTAGONISTS

2.2.1 Cetrotide®

On October 1, 2013, we announced that we had completed the transactions contemplated by the transfer and service agreement and concurrent agreements with various partners and licensees with respect to the manufacturing rights for Cetrotide®, currently marketed by a subsidiary of Merck KGaA of Darmstadt, Germany ("Merck Serono") for therapeutic use as part of in vitro fertilization programs. The principal outcome of these agreements is the transfer of manufacturing rights and the grant of a license to Merck Serono for the manufacture, testing, assembling, packaging, storage and release of Cetrotide® in all territories in exchange for a non-refundable one-time payment of €2.5 million (approximately $3.3 million). In addition, we also entered into a transitional services agreement with Merck KGaA under which the Company will, during a 36-month period, provide various transition services to assist Merck KGaA in assuming responsibility for the manufacturing of Cetrotide® in consideration for the payment of a monthly fee to the Company throughout such period.

2.2.2 Ozarelix

Ozarelix is a modified LHRH antagonist which is a linear decapeptide sequence. Ozarelix is a fourth-generation LHRH antagonist designed to extend the suppression of testosterone levels, which does not require a sophisticated depot formulation for long-lasting activity.

On August 12, 2004, we entered into a licensing and collaboration agreement with Spectrum for ozarelix and its potential to treat hormone-dependent cancers as well as benign proliferative disorders, such as BPH and endometriosis for all potential indications in North America (including Canada and Mexico) and India while keeping the rights for the rest of the world. In addition, Spectrum is entitled to receive 50% of upfront and milestone payments and royalties received from our Japanese partner, Nippon Kayaku, that are generated in the Japanese market for oncological indications. In November 2010, this agreement with Spectrum was amended. Under the terms of the amended agreement, Spectrum is entitled to use our patent rights and know-how to develop, use, make, have made, sell, offer for sale, have sold, import, export and commercialize ozarelix in all worldwide territories except Japan, Korea, Indonesia, Malaysia, the Philippines and Singapore. Under the terms of the amended agreement, Spectrum granted, as further consideration, 326,956 shares of its common stock, with an equivalent fair value at the time of approximately $1,263,000, as an upfront nonrefundable license fee payment to us. Also per the amended agreement, we will be entitled to receive a total of approximately $22,765,000 in cash payments, as well as approximately $670,000 in Spectrum common stock, upon achieving certain regulatory milestones in various markets. Furthermore, we will be entitled to receive royalties (scale-up royalties from high single to low double-digit) on future net sales of ozarelix products in the named territories.

On August 3, 2006, we announced a licensing and collaboration agreement with Nippon Kayaku for ozarelix. Under the terms of the agreement, we granted Nippon Kayaku an exclusive license to develop and market ozarelix for all potential oncological indications in Japan. In return, we received an upfront payment upon signature and are eligible to receive payments upon achievement of certain development and regulatory milestones, in addition to low double-digit royalties on potential net sales. Spectrum is entitled to receive 50% of the upfront, milestone payments and royalties received from Nippon Kayaku.

During the third quarter of 2008, we entered into a commercialization agreement with Handok for ozarelix (BPH indication) for the Korean market.

2.2.2.1 Prostate Cancer Clinical Trials

In August 2006, we announced positive Phase 2 results for ozarelix in hormone-dependent inoperable prostate cancer. This open-label, randomized-controlled dose-finding trial enrolled 64 patients receiving different IM dosage regimens of ozarelix to assess its safety and efficacy. The study achieved its primary endpoint of defining a tolerable dosage regimen of ozarelix that would ensure continuous suppression of testosterone at castration level for a three-month test period. A secondary efficacy endpoint aimed at assessing tumor response as determined by a 50% or greater reduction of serum PSA level, compared to baseline, was also achieved. The best results regarding the primary endpoint of continuous suppression were obtained with a dose of 130 mg per cycle where all patients remained suppressed to castration until at least day 85. In patients with continuous testosterone suppression below castration level, tumor response as measured by PSA levels was 97%.

A Phase 2 trial for the treatment of prostate cancer is currently ongoing with our partner, Spectrum. This is an international, multicenter, open-label, randomized study assessing the safety and efficacy of a monthly dosing regimen of ozarelix versus goserelin depot in men with prostate cancer (source: www.clinicaltrials.gov).

RAW MATERIALS

Raw materials and supplies are generally available in quantities adequate to meet the needs of our business. We will be dependent on third-party manufacturers for the pharmaceutical products that we will market. An interruption in the availability of certain raw materials or ingredients, or significant increases in the prices paid by us for them, could have a material adverse effect on our business, financial condition, liquidity and operating results.

DISTRIBUTION

Regarding MACRILEN™, throughout the remainder of 2014, we expect to advance the pre-launch activities related to the initial commercialization of this product for the evaluation of AGHD in the U.S. market. As noted above, our NDA is currently under substantive review by the FDA. Subject to the successful review and acceptance of our NDA, we expect to make MACRILEN™ available by prescription in the U.S. as soon as commercially practicable following final regulatory approval.

We intend to build a commercial infrastructure necessary to access the physicians who perform the majority of AGHD tests (endocrinologists) along with the major centers of AGHD influence. Commercial initiatives are likely to include the targeted selection, hiring and deployment of a contracted sales force by the end of 2014. The marketing efforts of our sales force will target endocrinology specialists of AGHD. We believe these efforts should enable the realization of a substantial portion of the potential commercial opportunity for MACRILEN™.

We are evaluating the possible final distribution channels for MACRILEN™, however, we expect that MACRILEN™ will be accessed through a mixture of specialty pharmacies, hospital pharmacies, wholesalers and other secondary channels.

To date, we have established an agreement with a contract manufacturer for the commercial supply of the product and expect to use a third-party logistics provider for key services related to logistics, warehousing and inventory management.

We continue to evaluate the potential to commercialize MACRILEN™ in other geographic territories, including Canada and Europe.

REGULATORY COMPLIANCE

Governmental authorities in Canada, the United States, Europe and other countries extensively regulate the preclinical and clinical testing, manufacturing, labeling, storage, record keeping, advertising, promotion, export, marketing and distribution, among other things, of our product candidates. Under the laws of the United States, the countries of the EU, and other countries, we and the institutions at which we sponsor research are subject to obligations to ensure that our clinical trials are conducted in accordance with Good Clinical Practices ("GCP") guidelines and the investigational plan and protocols contained in an IND application, or comparable foreign regulatory submission. The Japanese regulatory process for approval of new drugs is similar to the FDA approval process described below except that Japanese regulatory authorities request bridging studies to verify that foreign clinical data are applicable to Japanese patients and also require the tests to determine appropriate dosages for Japanese patients to be conducted on Japanese patient volunteers. Due to these requirements, delays of two to three years in introducing a drug developed outside of Japan to the Japanese market are possible. Set forth below is a brief summary of the material governmental regulations

affecting the Company in the major markets in which we intend to market our products.

Canada

In Canada, the Canadian Therapeutic Products Directorate is the Canadian federal authority that regulates pharmaceutical drugs and medical devices for human use. Prior to being given market authorization, a manufacturer must present substantive scientific evidence of a product's safety, efficacy and quality as required by the Food and Drugs Act and other legislation and regulations. The requirements for the development and sale of pharmaceutical drugs in Canada are substantially similar to those in the United States, which are described below.

United States

In the United States, the FDA under the Federal Food, Drug, and Cosmetic Act, the Public Health Service Act and other federal statutes and regulations, subject pharmaceutical products to rigorous review.

In order to obtain approval of a new product from the FDA, we must, among other requirements, submit proof of safety and efficacy as well as detailed information on the manufacture and composition of the product. In most cases, this proof entails extensive preclinical, clinical, and laboratory tests. Before approving a new drug or marketing application, the FDA also typically conducts pre-approval inspections of the company, its CROs and/or its clinical trial sites to ensure that clinical, safety, quality control, and other regulated activities are compliant with GCP, or Good Laboratory Practices ("GLP"), for specific non-clinical toxicology studies. Manufacturing facilities used to produce a product are also subject to ongoing inspection by the FDA. The FDA may also require confirmatory trials, post-marketing testing, and extra surveillance to monitor the effects of approved products, or place conditions on any approvals that could restrict the commercial applications of these products. Once approved, the labeling, advertising, promotion, marketing, and distribution of a drug or biologic product must be in compliance with FDA regulatory requirements.

The first stage required for ultimate FDA approval of a new biologic or drug involves completion of preclinical studies and the submission of the results of these studies to the FDA. This, together with proposed clinical protocols, manufacturing information, analytical data, and other information in an IND, must become effective before human clinical trials may commence. Preclinical studies involve laboratory evaluation of product characteristics and animal studies to assess the efficacy and safety of the product. The FDA regulates preclinical studies under a series of regulations called the current GLP regulations. If the sponsor violates these regulations, the FDA may require that the sponsor replicate those studies.

After the IND becomes effective, a sponsor may commence human clinical trials. The sponsor typically conducts human clinical trials in three sequential phases, but the phases may overlap. In Phase 1 trials, the sponsor tests the product in a small number of patients or healthy volunteers, primarily for safety at one or more doses. Phase 1 trials in cancer are often conducted with patients who have end-stage or metastatic cancer. In Phase 2, in addition to safety, the sponsor evaluates the efficacy of the product in a patient population somewhat larger than Phase 1 trials. Phase 3 trials typically involve additional testing for safety and clinical efficacy in an expanded population at geographically dispersed test sites. The sponsor must submit to the FDA a clinical plan, or "protocol", accompanied by the approval of the institutions participating in the trials, prior to commencement of each clinical trial. The FDA may order the temporary or permanent discontinuation of a clinical trial at any time. In the case of product candidates for cancer, the initial human testing may be done in patients with the disease rather than in healthy volunteers. Because these patients are already afflicted with the target disease, such studies may provide results traditionally obtained in Phase 2 studies. Accordingly, these studies are often referred to as "Phase 1/2" studies. Even if patients participate in initial human testing and a Phase 1/2 study is carried out, the sponsor is still responsible for obtaining all the data usually obtained in both Phase 1 and Phase 2 studies.

The sponsor must submit to the FDA the results of the preclinical and clinical testing, together with, among other things, detailed information on the manufacture and composition of the product, in the form of an NDA or, in the case of a biologic, a Biologics License Applications ("BLA"). In a process that can take a year or more, the FDA reviews this application and, when and if it decides that adequate data are available to show that the new compound is both safe and effective for a particular indication and that other applicable requirements have been met, approves the drug or biologic for marketing. The amount of time taken for this approval process is a function of a number of variables, including the quality of the submission and studies presented and the potential contribution that the compound will make in improving the treatment of the disease in question.

Orphan-drug designation is granted by the FDA Office of Orphan Drug Products to novel drugs or biologics that treat a rare disease or condition affecting fewer than 200,000 patients in the U.S. The designation provides the drug developer with a seven-year period of U.S. marketing exclusivity if the drug is the first of its type approved for the specified indication or if it demonstrates superior safety, efficacy or a major contribution to patient care versus another drug of its type previously granted the designation for the same indication. We have been granted orphan drug designations for zoptarelin doxorubicin for the treatment of advanced ovarian cancer and for MACRILEN™ for the evaluation of growth hormone deficiency.

Under the Hatch-Waxman Act, newly-approved drugs and indications may benefit from a statutory period of non-patent data exclusivity. The Hatch-Waxman Act provides five-year data exclusivity to the first applicant to gain approval of an NDA for a new chemical entity, or NCE, meaning that the FDA has not previously approved any other drug containing the same active pharmaceutical ingredient, or active moiety. Although protection under the Hatch-Waxman Act will not prevent the submission or approval of another full NDA, such an NDA applicant would be required to conduct its own preclinical and adequate, well controlled clinical trials to demonstrate safety and effectiveness.

The Hatch-Waxman Act also provides three years of data exclusivity for the approval of new and supplemental NDAs, including Section 505(b)(2) applications, for, among other things, new indications, dosage forms, routes of administration, or strengths of an existing drug, or for a new use, if new clinical investigations that were conducted or sponsored by the applicant are determined by the FDA to be essential to the approval of the application. This exclusivity, which is sometimes referred to as clinical investigation exclusivity, would not prevent the approval of another application if the applicant has conducted its own adequate, well-controlled clinical trials demonstrating safety and efficacy, nor would it prevent approval of a generic product that did not incorporate the exclusivity-protected changes of the approved drug product.

The labeling, advertising, promotion, marketing, and distribution of a drug or biologic product must be in compliance with FDA regulatory requirements. Failure to comply with applicable requirements can lead to the FDA demanding that production and shipment cease and, in some cases, that the manufacturer recall products, or to enforcement actions that can include seizures, injunctions, and criminal prosecution. These failures can also lead to FDA withdrawal of approval to market a product.

European Union

Medicines can be authorized in the EU by using either the centralized authorization procedure or national authorization procedures.

Centralized procedure

The EU has implemented a centralized procedure coordinated by the EMA for the approval of human medicines, which results in a single marketing authorization issued by the European Commission that is valid across the EU, as well as Iceland, Liechtenstein and Norway. The centralized procedure is compulsory for human medicines that are derived from biotechnology processes, such as genetic engineering, that contain a new active substance indicated for the treatment of certain diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative disorders or autoimmune diseases and other immune dysfunctions, and designated orphan medicines. For medicines that do not fall within these categories, an applicant has the option of submitting an application for a centralized marketing authorization to the EMA, as long as the medicine concerned is a significant therapeutic, scientific or technical innovation, or if its authorization would be in the interest of public health.

National authorization procedures

There are also two other possible routes to authorize medicinal products in several EU countries, which are available for investigational drug products that fall outside the scope of the centralized procedure:

Decentralized procedure. Using the decentralized procedure, an applicant may apply for simultaneous authorization in more than one EU country of medicinal products that have not yet been authorized in any EU country and that do not fall within the mandatory scope of the centralized procedure.

The application will be reviewed by a selected Reference Member State ("RMS"). The Marketing Authorization granted by the RMS will then be recognized by the other Member States involved in this procedure.

Mutual recognition procedure. In the mutual recognition procedure, a medicine is first authorized in one EU Member State, in accordance with the national procedures of that country. Following this, further marketing authorizations can be sought from other EU countries in a procedure whereby the countries concerned agree to recognize the validity of the original, national marketing authorization.

For more information about the regulatory risks associated with the Company's business operations, see "Item 3. – Key Information – Risk Factors".

DRUG DISCOVERY

There is an increasing demand on the world market for active substances. Our internal drug discovery unit provides an important prerequisite for the provision of new patented active substances, which can then be developed further or licensed to third parties.

Our drug discovery unit concentrates on the search for active substances for innovative targets, which open the door to the introduction of new therapeutic approaches. Further, this unit searches for new active substances having improved properties for clinically validated targets for which drugs are already being used in humans and which produce inadequate effects, cause severe side effects, are not economical or are not available in a patient-friendly form.

To this end, we possess an original substance library for the discovery of active compounds with a comprehensive range of promising natural substances which can serve as models for the construction of synthetic molecules. The initial tests involve 120,000 samples from our internal substance library in the form of high-throughput screening. The "hits", which are the first active compounds found in the library, are tested further and built up specifically into potential lead structures. Based on two to three lead structures, they are then optimized in a further step to potential development candidates.

INTELLECTUAL PROPERTY – PATENTS

We believe that we have a solid intellectual property portfolio that covers compounds, manufacturing processes, compositions and methods of medical use for our lead drugs and drug candidates. Our patent portfolio consists of approximately 50 owned and in-licensed patent families (issued, granted or pending in the United States, Europe and other jurisdictions). Independent of the original patent expiry date, additional exclusivity is possible in the United States, Europe and several other countries by data protection for new chemical entities or by orphan drug designation. In addition, in the United States, Europe and certain other jurisdictions the terms of a patent covering an approved drug can be extended by patent term extension or supplementary protection certificate.

In the United States, the patent term of a patent that covers an FDA-approved drug may also be eligible for patent term extension, which permits patent term restoration as compensation for the patent term lost during the FDA regulatory review process. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the expiration of the patent, in which the patentee may file an application for yearly interim extensions within five years if the patent will expire and the FDA has not yet approved the NDA. The length of the patent term extension is related to the length of time the drug is under regulatory review. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended.

Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug. In these jurisdictions, however, no interim extensions exist and the marketing approval must be granted before the patent expires. In the future, if and when our pharmaceutical products receive FDA approval, we expect to apply for patent term extensions on patents covering those products. While we anticipate that any such applications for patent term extensions will likely be granted, we cannot predict the precise length of the time for which such patent terms would be extended in the United States, Europe or other jurisdictions. If we are not able to secure patent term extensions on patents covering our products for meaningful periods of additional time, we may not achieve or sustain profitability, which would adversely affect our business.

Of the issued or granted patents, the protective rights described below form the core of our patent portfolio with regard to our lead drugs and drug candidates.

Zoptarelin doxorubicin:

- U.S. patent 5,843,903 provides protection in the United States for the compound zoptarelin doxorubicin and other related targeted cytotoxic anthracycline analogs, pharmaceutical compositions comprising the compounds as well as their medical use for the treatment of cancer. This U.S. patent expires in November 2015. A patent term extension of up to five years may be possible.
- European patent 0 863 917 B1 provides protection in Europe for the compound zoptarelin doxorubicin and other related targeted cytotoxic anthracycline analogs, pharmaceutical compositions comprising the compounds as well as their medical use for the treatment of tumors. This European patent expires in November 2016. A patent term extension of up to five years may be possible in case approval has been achieved prior to patent expiration.
- Japanese patent 3 987 575 provides protection in Japan for the compound zoptarelin doxorubicin and other related targeted cytotoxic anthracycline analogs, pharmaceutical compositions comprising the compounds as well as their medical use for the treatment of tumors. This Japanese patent expires in November 2016. A patent term extension of up to five years may be possible in case approval has been achieved prior to patent expiration.

Macimorelin:

- U.S. patent 6,861,409 protects the compound macimorelin and U.S. patent 7,297,681 protects other related growth hormone

secretagogue compounds, each also protecting pharmaceutical compositions comprising the compounds as well as their medical use for elevating the plasma level of growth hormone. U.S. patent 6,861,409 and U.S. patent 7,297,681 both expire in August 2022. A patent term extension of up to five years may be possible.

- European patent 1 289 951 protects the compound macimorelin and European patent 1 344 773 protects other related growth hormone secretagogue compounds, pharmaceutical compositions comprising the compounds as well as their medical use for elevating the plasma level of growth hormone. EP patent 1 289 951 and EP patent 1 344 773 both expire in June 2021. A patent term extension of up to five years by SPC may be possible in case approval has been achieved prior to patent expiration.
- Japanese patent 3 522 265 protects the compound macimorelin and pharmaceutical compositions comprising the compounds as well as their medical use for elevating the plasma level of growth hormone. This Japanese patent expires in June 2021. A patent term extension of up to five years may be possible in case approval has been achieved prior to patent expiration.
- Canadian patent 2,407,659 protects the compound macimorelin and pharmaceutical compositions comprising the compounds as well as their medical use for elevating the plasma level of growth hormone. This Canadian patent expires in June 2021.
- U.S. patent 8,192,719 protects a method of assessing pituitary-related growth hormone deficiency in a human or animal subject comprising an oral administration of the compound macimorelin and determination of the level of growth hormone in the sample and assessing whether the level of growth hormone in the sample is indicative of growth hormone deficiency. This U.S. patent 8,192,719 expires in October 2027.
- European patent 1 984 744 protects a method of assessing pituitary-related growth hormone deficiency by oral administration of macimorelin. The European patent 1 984 744 expires in February 2027.
- Japanese patent 4 852 728 protects a method of assessing pituitary-related growth hormone deficiency by oral administration of macimorelin. The Japanese patent 4 852 728 expires in February 2027.

AEZS-120:

- European patent 2 092 067 B1 provides protection in Europe for microorganisms as carriers of heterogeneous nucleotide sequences coding for antigens and protein toxins, a process of manufacturing thereof as well as corresponding plasmids or expression vectors, useful as medicaments, in particular as tumor vaccines for the treatment of various tumors. This European patent expires in November 2027. A patent term extension of up to five years may be possible in case approval has been achieved prior to patent expiration.
- U.S. and Japanese patent applications (both filed in November 2007) recently received a Notice of Allowance. Granted patents will expire in November 2027.

Ozarelix:

- U.S. patent 6,627,609 provides protection in the United States for the compound ozarelix and related third-generation LHRH antagonists and pharmaceutical compositions comprising them. This U.S. patent will expire in March 2020. A patent term extension of up to five years may be possible.
- European patent 1 163 264 provides protection in Europe for the compound ozarelix and related third-generation LHRH antagonists and pharmaceutical compositions comprising them. This European patent will expire in March 2020. A SPC of up to five years may be possible in case approval has been achieved prior to patent expiration.
- Japanese patent 3 801 867 provides protection in Japan for the compound ozarelix and related third-generation LHRH antagonists and pharmaceutical compositions comprising them. This Japanese patent will expire in March 2020. A patent term extension of up to five years may be possible in case approval has been achieved prior to patent expiration.

Erk/PI3K:

- U.S. patent 8,202,883 protects compound AEZS-129. This U.S. patent will expire in May 2029 (including patent term adjustment ("PTA"). A patent term extension of up to five years may be possible.
- U.S. patent 8,507,486 protects compound AEZS-136. This U.S. patent will expire in May 2028. A patent term extension of up to five years may be possible.
- U.S. patent 8,536,332 protects methods of treatment for compound AEZS-129. This U.S. patent will expire in May 2028. A patent term extension of up to five years may be possible.

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- U.S. patent 8,604,196 protects methods of treatment for compound AEZS-136. This U.S. patent will expire in May 2028 and is subject to a terminal disclaimer based on US 8,507,486 (07/04Z/2). A patent term extension of up to five years may be possible.
- U.S. patent application US-2012-0258080 seeks protection for compound AEZS-134 as well as methods of treatment for this compound. When granted, the U.S. patent would expire in April 2032. A patent term extension of up to five years may be possible.
- European Patent Application EP2,164,849 seeks protection for compounds AEZS-129 and -136 as well as methods of treatment for these compounds. When granted, the EP patent would expire in May 2028. A SPC of up to five years may be possible in case approval has been achieved prior to patent expiration.
- European Patent Application No. EP2,694,067 seeks protection for compound AEZS-134 as well as methods of treatment for this compound. When granted, the EP patent would expire in April 2032. A SPC of up to five years may be possible in case approval has been achieved prior to patent expiration.
- Japanese Patent Application No. 2010-506945 seeks protection for compound AEZS-129 as well as methods of treatment for this compound. When granted, the JP patent would expire in May 2028. A SPC of up to five years may be possible in case approval has been achieved prior to patent expiration.
- Japanese Patent Application No. 2014-6832 seeks protection for compounds AEZS-136 as well as methods of treatment for this compound. When granted, the JP patent would expire in May 2028. A SPC of up to five years may be possible in case approval has been achieved prior to patent expiration.
- Japanese patent application based on PCT/EP2012/056138 seeks protection for compound AEZS-134 as well as methods of treatment for this compound. When granted, the JP patent would expire in April 2032. A SPC of up to five years may be possible in case approval has been achieved prior to patent expiration.

Disorazol Z - LHRH conjugates (AEZS-138):

- U.S. patent 7,741,277 protects compound AEZS-138 (disorazole Z - LHRH conjugate). This U.S. patent will expire in January 2028 (including PTA). A patent term extension of up to five years may be possible.
- U.S. patent 8,470,776 protects methods of treatment for compound AEZS-138 (disorazole Z - LHRH conjugate). This U.S. patent will expire in February 2029 (including PTA). A patent term extension of up to five years may be possible.
- European patent application 2,066,679 protects compound AEZS-138 (disorazole Z - LHRH conjugate) as well as methods of treatment for this compound. When granted, this EP patent will expire in September 2027. A SPC of up to five years may be possible in case approval has been achieved prior to patent expiration.
- Japanese patent 5,340,155 protects compound AEZS-138 (disorazole Z - LHRH conjugate) as well as methods of treatment for this compound. This JP patent will expire in September 2027. A SPC of up to five years may be possible in case approval has been achieved prior to patent expiration.

Overview of important granted patents in the United States, Europe and Japan:

Patent No.	Title	Country	Expiry Date
Zoptarelin doxorubicin			
U.S. 5,843,903	Targeted cytotoxic anthracycline analogs	United States	2015-11-27
EP 0 863 917	Targeted cytotoxic anthracycline analogs	Europe	2016-11-14
JP 3 987 575	Targeted cytotoxic anthracycline analogs	Japan	2016-11-14
Macimorelin			
U.S. 6,861,409	Growth hormone secretagogues	United States	2022-08-01
EP 1 289 951	Growth hormone secretagogues	Germany, United Kingdom, France, Switzerland and others	2021-06-13
JP 3 522 265	Growth hormone secretagogues	Japan	2021-06-13
CA 2,407,659	Growth hormone secretagogues	Canada	2021-06-13
U.S. 8,192,719	Method and kit to diagnose growth hormone deficiency	United States	2027-10-12

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Patent No.	Title	Country	Expiry Date
EP 1 984 744	Method and kit to diagnose growth hormone deficiency	Europe	2027-02-19
JP 4 852 728	Method and kit to diagnose growth hormone deficiency	Japan	2027-02-19
AEZS-120			
EP 2 092 067	Microorganisms as carriers of nucleotide sequences	Europe	2027-11-13
AEZS-112			
U.S. 7,365,081	Indole derivatives and their use as medicaments	United States	2017-09-08
EP 1 309 585	Indole derivatives and their use as medicaments	Germany, United Kingdom, France, Switzerland and others	2021-07-26
Ozarelix			
U.S. 6,627,609	LHRH antagonists having improved solubility properties	United States	2020-03-14
EP 1 163 264	LHRH antagonists having improved solubility properties	Germany, United Kingdom, France, Switzerland and others	2020-03-11
JP 3 801 867	LHRH antagonists having improved solubility properties	Japan	2020-03-11
AEZS-129			
U.S. 8,202,883	Novel Pyridopyrazine Derivatives, Process of Manufacturing and Uses thereof	United States	2029-05-29*
U.S. 8,536,332	Novel Pyridopyrazine Derivatives, Process of Manufacturing and Uses thereof	United States	2028-05-09
EP Patent Appl. EP 2,164,849	Novel Pyridopyrazine Derivatives, Process of Manufacturing and Uses thereof	Europe	2028-05-09
JP Patent Appl. JP 2010-506945	Novel Pyridopyrazine Derivatives, Process of Manufacturing and Uses thereof	Japan	2028-05-09
AEZS-136			
U.S. 8,507,486	Novel Pyridopyrazine Derivatives, Process of Manufacturing and Uses thereof	United States	2028-05-09
U.S. 8,604,196	Novel Pyridopyrazine Derivatives, Process of Manufacturing and Uses thereof	United States	2028-05-09 (term. disclaimer)
EP Patent Appl. EP 2,164,849	Novel Pyridopyrazine Derivatives, Process of Manufacturing and Uses thereof	Europe	2028-05-09
JP Patent Appl. JP 2014-6832	Novel Pyridopyrazine Derivatives, Process of Manufacturing and Uses thereof	Japan	2028-05-09
AEZS-134			
	Pyridopyrazine Derivatives and their Use	United States	2032-04-04

U.S. Patent Appl. US 2012-0258080 EP Patent Appl. EP 2,694,067	Pyridopyrazine Derivatives and their Use	Europe	2032-04-04
JP pat. appl. based on PCT/EP2012/056138	Pyridopyrazine Derivatives and their Use	Japan	2032-04-04
AEZS-138			
U.S.7,741,277	Conjugates of Disorazoles and Derivatives, Process of Manufacturing and Uses thereof	United States	2028-01-19*
U.S. 8,470,776	Conjugates of Disorazoles and Derivatives, Process of Manufacturing and Uses thereof	United States	2029-02-02*
EP Patent Appl. EP 2,066,679	Conjugates of Disorazoles and Derivatives, Process of Manufacturing and Uses thereof	Europe	2027-09-06
JP 5,340,155	Conjugates of Disorazoles and Derivatives, Process of Manufacturing and Uses thereof	Japan	2027-09-06

* Includes Patent Term Extension.

C. Organizational structure

The following chart presents our corporate structure, the jurisdiction of incorporation of our direct and indirect subsidiaries and the percentage of shares that we held in those subsidiaries as at December 31, 2013.

Aeterna Zentaris Inc.
(Canada)

100%

Aeterna Zentaris GmbH
(Germany)

100%

Zentaris IVF GmbH
(Germany)

100%

Aeterna Zentaris, Inc.
(Delaware)

D. Property, plants and equipment

Our corporate head office is located in Quebec City, Province of Quebec, Canada. The following table sets forth information with respect to our main facilities as at December 31, 2013.

Location	Use of space	Square Footage	Type of interest
1405 du Parc Technologique Blvd., Quebec City (Quebec), Canada	Fully occupied for management, R&D and administration	3,561	Leased
25 Mountainview Blvd., Suite 203, Basking Ridge, NJ 07920	Fully occupied for management, R&D and administration	3,188	Leased
Weismüllerstr. 50 D-60314 Frankfurt-am-Main, Germany	Fully occupied for management, R&D, business development and administration	46,465	Leased

Item 4A Unresolved Staff Comments

None.

Item 5. Operating and Financial Review and Prospects

Key Developments

MACRILEN™

On January 6, 2014, we announced that the FDA had accepted for substantive review our New Drug Application ("NDA") for our orally available peptidomimetic ghrelin receptor agonist with growth hormone secretagogue activity, MACRILEN™, for the evaluation of adult growth hormone deficiency ("AGHD"). The acceptance for filing of the NDA indicates that the FDA has determined that the application is sufficiently complete to permit a substantive review. The NDA, submitted on November 5, 2013, seeks approval for the commercialization of MACRILEN™, which, if approved, will be the first orally administered drug indicated for the evaluation of AGHD by evaluating the pituitary gland secretion of growth hormone in response to an oral dose of the product. The application is subject to a standard review and will have a Prescription Drug User Fee Act ("PDUFA") date of November 5, 2014. The PDUFA date is the goal date for the FDA to complete its review of the NDA. MACRILEN™ benefits from patent protection covering major markets; in particular, the product is protected in the U.S. at least until October 2027. Phase 3 data have demonstrated the compound to be well tolerated, with accuracy comparable to available intravenous and intramuscular testing procedures.

Zoptarelin Doxorubicin

On April 10, 2013 we announced the signing of a co-development and profit sharing agreement with Ergomed Clinical Research Ltd. ("Ergomed") as the contract clinical development organization for the Phase 3 ZoptEC (Zoptarelin doxorubicin in Endometrial Cancer) trial in women with locally advanced, recurrent or metastatic endometrial cancer who have progressed and who have received one chemotherapeutic regimen with platinum and taxane (either as adjuvant or first-line treatment). The ZoptEC trial is an open-label, randomized, multicenter trial conducted in North America, Europe and Israel under a Special Protocol Assessment with the FDA. The trial compares zoptarelin doxorubicin with doxorubicin as second line therapy and will involve approximately 500 patients. Patient dosing was initiated in July 2013, and the primary efficacy endpoint of the ZoptEC trial is improvement in median Overall Survival.

Under the terms of the agreement, Ergomed has agreed to assume 30% (up to $10 million) of the clinical and regulatory costs for this trial, which are estimated at approximately $30 million over the course of the study. Ergomed will be entitled to receive an agreed upon single-digit percentage of any net income received by us for zoptarelin doxorubicin in this indication, up to a specified maximum amount.

On June 3, 2013, we announced encouraging final data for the Phase 1 portion of the ongoing Phase 1/2 trial in men with castration- and taxane-resistant prostate cancer with zoptarelin doxorubicin. Data were presented at the American Society of Clinical Oncology Annual Meeting in Chicago by the principal investigator, Jacek Pinski, MD, PhD, of the University of Southern California's Norris Comprehensive Cancer Center. In general, zoptarelin doxorubicin was well tolerated and demonstrated promising evidence of its anti-tumor activity in this heavily pretreated population. Among the 15 evaluable patients with measurable disease, ten achieved stable disease, and a drop in Prostatic Specific Antigen was noted in three patients. The maximum tolerated dose ("MTD") of zoptarelin doxorubicin in this indication was established at 210 mg/m^2, which is below the MTD reported in women with refractory endometrial and ovarian cancer. The Phase 2 portion of this trial in prostate cancer is ongoing.

Cetrotide® Manufacturing Rights and Discontinued Operations

On October 1, 2013, we announced that we had successfully completed the transactions contemplated by the transfer and service agreement and concurrent agreements with various partners and licensees with respect to the manufacturing rights for Cetrotide®, currently marketed by a subsidiary of Merck KGaA of Darmstadt, Germany ("Merck Serono") for therapeutic use as part of in vitro fertilization programs. The principal outcome of these agreements is the transfer of manufacturing rights and the grant of a license to Merck Serono for the manufacture, testing, assembling, packaging, storage and release of Cetrotide® in all territories (the "Cetrotide® Business") in exchange for a non-refundable, one-time payment of €2.5 million (approximately $3.3 million).

The Cetrotide® Business has been presented in our consolidated financial statements as a discontinued operation. As such, relevant amounts impacting elements of our comprehensive income (loss) and cash flows have been retroactively reclassified to reflect the Cetrotide® Business as a discontinued operation and are discussed separately

from continuing operations in this MD&A.

Perifosine

On March 11, 2013, we announced that the Phase 3 trial in multiple myeloma was discontinued after an interim analysis by an independent Data Safety Monitoring Board reported that it was highly unlikely the study would achieve a significant difference in its primary endpoint of progression-free survival. We therefore decided not to make any further investment in the development of perifosine.

Appointments to Executive Management Team

On April 15, 2013, we announced the appointment of David Dodd as our President, Chief Executive Officer ("CEO") and director of the Company. Mr. Dodd's executive management experience in the pharmaceutical and biotechnology industries spans more than 35 years. Prior to joining our Company, Mr. Dodd was President, CEO and Chairman of BioReliance Corporation, a leading provider of biological safety and related testing services, and President, CEO and director of Serologicals Corporation. Mr. Dodd also held the roles of President and CEO of Solvay Pharmaceuticals, Inc. and of Chairman of its subsidiary, Unimed Pharmaceuticals, Inc., and held various senior management positions at Wyeth-Ayerst Laboratories, the Mead Johnson Laboratories Division at Bristol-Myers Squibb and at Abbott Laboratories. Mr. Dodd holds a Master's degree from Georgia State University and completed the Harvard Business School Advanced Management Program.

On November 1, 2013, we announced the appointment of Jude Dinges as our Senior Vice President and Chief Commercial Officer. Mr. Dinges is responsible for all activities regarding the potential commercial launch of MACRILEN™ in AGHD, as well as for identifying future commercial opportunities. Mr. Dinges began his career nearly 30 years ago at Bristol Laboratories and later at Merck & Co. in training, sales, management, marketing and market development and was a key contributor to the successful launch of brands such as Cozaar®, Fosamax®, Singulair®, Maxalt®, Vioxx®, and Vytorin®. Mr. Dinges joined Novartis Pharmaceuticals in 2006, overseeing the launch of Tekturna®, and in 2008 became the Respiratory & Infectious Disease Specialty Medicines Director. In 2009, he joined Amgen Inc. as Executive Director of Region Sales, Bone Health Business Unit.

On January 3, 2014, we announced the appointment of Richard Sachse, MD, PhD, as our Senior Vice President, Chief Scientific Officer and Managing Director. Dr. Sachse, who is based in Frankfurt, holds a degree in medicine from the Friedrich-Alexander-University Erlangen and a board certification in Clinical Pharmacology and has over 20 years' experience as a physician and scientist. He has extensive expertise in a variety of different therapeutic areas, including endocrinology and oncology. In addition to registration studies, Dr. Sachse is especially experienced in the design and implementation of translational programs to bridge research programs to the clinic, as well as in the design and implementation of clinical pharmacology programs, including all required profiling studies and activities, enabling successful registration of products at the international level. Before joining Aeterna Zentaris, Dr. Sachse was Vice President and Head of Global Translational Medicine at Boehringer Ingelheim. From 1996 to 2000, he was International Project Leader at the Bayer AG Institute for Clinical Pharmacology and Principal Investigator at the Bayer Clinical Pharmacology Unit. From 2001 to 2006, Dr. Sachse held a variety of management positions within early and late phase clinical development programs, including responsibilities for completed Phase 3 programs leading to successful New Drug Application / Marketing Authorization Application submissions. In 2007, he became Senior Director, Head of Experimental Medicine, at UCB in Belgium, before being appointed Vice President, Head of Global Translational Medicine, at Boehringer Ingelheim in 2010.

Corporate Developments

"At-the-Market" Issuance Program

Between May 22, 2013 and December 31, 2013, we sold a total of approximately 1.7 million common shares under our At The-Market ("ATM") sales program at an average price of $1.76 per share, resulting in aggregate gross proceeds of approximately $3.0 million. This ATM sales program allows the Company to sell, at market prices prevailing at the time of sale, up to a maximum of 2.5 million of our common shares through ATM issuances on the NASDAQ for aggregate gross proceeds not to exceed $4.6 million. Between January 1, 2014 and March 20, 2014, we issued a total of 0.2 million common shares under this ATM sales program for aggregate gross proceeds of $0.3 million.

Registered Direct Offering

On July 30, 2013, we completed a registered direct offering of 5.2 million units at a purchase price of $1.50 per unit, generating net proceeds of approximately $7.0 million. Each unit consisted of one common share and 0.5 of a warrant to purchase one common share. Each warrant is exercisable at any time after January 30, 2014 for a period of five years from the date of issuance at an exercise price of $1.85 per share.

Public Offerings

On November 25, 2013 we completed a public offering of 13.1 million units, generating net proceeds of approximately $13.7 million. Each unit consisted of one common share and one whole warrant to purchase one common share, at a purchase price of $1.15 per unit. Each warrant is exercisable for a period of five years at an original exercise price of $1.60 per share, subject to certain anti-dilution provisions.

Subsequent to year-end, on January 14, 2014, we completed a public offering of 11.0 million units, generating net proceeds of approximately $12.2 million, with each unit consisting of one common share and 0.8 of a warrant to purchase one common share, at a purchase price of $1.20 per unit. Each warrant is exercisable for a period of five years at an original exercise price of $1.25 per share, which is subject to certain anti-dilution provisions.

Listing Transfer to the NASDAQ Capital Market

On August 28, 2013, we announced that our request to transfer our listing to the NASDAQ Capital Market from the NASDAQ Global Market had been approved by the NASDAQ Listing Qualifications Staff. Our common shares continue to trade on the NASDAQ Capital Market, effective August 29, 2013.

Status of Our Drug Pipeline

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(1) Investigator-driven and sponsored.

(2) Phase 2 in ovarian cancer completed.

(3) Sponsored entirely by license partners.

We are focused on preparing for the launch of MACRILEN™ for the evaluation of AGHD in the U.S. and on advancing our ZoptEC Phase 3 program with zoptarelin doxorubicin in endometrial cancer, as discussed further below.

Regarding AEZS-120, which is a targeted, live recombinant oral tumor vaccine candidate, we are reviewing the development program and our available resources related to this compound.

Ozarelix, a modified luteinizing hormone-releasing hormone ("LHRH") receptor antagonist, with the potential to treat hormone-dependent cancers as well as benign proliferative endocrinological disorders, and perifosine, an oral AKT inhibitor which is being investigated as a potential treatment option for various cancer indications, no longer require significant investment from our Company, being licensed out to Spectrum Pharmaceuticals, Inc. and to Yakult Honsha Co., Ltd. ("Yakult"), respectively. Both partners are responsible for conducting and sponsoring all ongoing development.

As for our compounds in earlier stages of development, our Erk/PI3K inhibitors and our disorazol Z product candidates, as well as our discovery activities, are both under review as part of our focused initiative to optimize research and development ("R&D") activities. Our Erk/PI3K inhibitors are part of our kinase research program, comprising the investigation of different compounds for single Erk inhibition, single PI3K inhibition and dual Erk/PI3K kinase inhibition. Disorazol Z product candidates comprise AEZS-138, a novel cytotoxic hybrid based on the natural compound disorazol Z (AEZS-137), and the LHRH receptor agonist D-Lys6-LHRH. We currently do not expect to invest significantly in these projects, unless partnered and/or sponsored through strategic alliances.

Consolidated Statements of Comprehensive Income (Loss) Information

	Three-month periods ended December 31,		Years ended December 31,		
(in thousands, except share and per share data)	2013	2012	2013	2012	2011
	$	$	$	$	$
Revenues					
Sales	—	—	96	834	250
License fees and other	—	281	6,079	1,219	4,455
	—	281	6,175	2,053	4,705
Operating expenses					
Cost of sales	—	—	51	591	212
Research and development costs, net of refundable tax credits and grants	5,345	5,523	21,284	20,592	24,245
Selling, general and administrative expenses	2,627	2,877	12,316	10,606	11,955
	7,972	8,400	33,651	31,789	36,412
Loss from operations	(7,972)	(8,119)	(27,476)	(29,736)	(31,707)
Finance income	65	689	1,748	6,974	6,239
Finance costs	(2,689)	(700)	(1,512)	(382)	(8)
Net finance (costs) income	(2,624)	(11)	236	6,592	6,231
Loss before income taxes	(10,596)	(8,130)	(27,240)	(23,144)	(25,476)
Income tax expense	—	—	—	—	(1,104)
Net loss from continuing operations	(10,596)	(8,130)	(27,240)	(23,144)	(26,580)
Net income (loss) from discontinued operations	2,353	1,183	34,055	2,732	(487)
Net (loss) income	(8,243)	(6,947)	6,815	(20,412)	(27,067)
Other comprehensive (loss) income:					
Items that may be reclassified subsequently to profit or loss:					
Foreign currency translation adjustments	424	(204)			