

Endometrial Cancer Trial: Clinical Phase Completed



Aeterna Zentaris Announces Completion of Zoptrex™ Pivotal Phase 3 Clinical Trial in Advanced Endometrial Cancer; Expects to Report Top-Line Results in April 2017

CHARLESTON, S.C.-([BUSINESS WIRE](#))-Aeterna Zentaris Inc. (NASDAQ: AEZS) (TSX: AEZ) (the “Company”) today announced the occurrence of the 384th death in the pivotal Phase 3 ZoptEC (**Z**optarelin Doxorubicin in **E**ndometrial **C**ancer) study with Zoptrex™ (zoptarelin doxorubicin) in women with advanced, recurrent or metastatic endometrial cancer, representing the clinical endpoint of the study.

The Company currently expects to lock the clinical database and to report top-line results in April 2017. Zoptrex™ is the Company’s proposed tradename for

zoptarelin doxorubicin. The proposed tradename is subject to approval by the United States Food and Drug Administration (the "FDA").

Dr. Richard Sachse, the Company's Chief Scientific Officer, stated, "We are pleased to announce the completion of the clinical phase of our pivotal Phase 3 clinical study of Zoptrex™, which was conducted under a Special Protocol Assessment with the FDA. Reaching this important milestone took longer than we anticipated because the rate of events slowed significantly during the past year. As previously reported, the study was fully enrolled in June 2015 and the final dosing occurred in January 2016. Therefore, a significant number of patients survived more than 18 months since enrollment in the study. We are thankful that these patients continued to survive a devastating disease and are hopeful that their lives are continuing successfully. We are close to locking the clinical database and are focused on producing the top-line results of the study. Currently, we expect to release top-line results in April 2017."

David A. Dodd, President and Chief Executive Officer of the Company stated, "With the completion of the clinical portion of this trial, we will now focus on analyzing the data and, if warranted by the results, submitting a new drug application later this year. There is a significant unmet medical need for a treatment for women with advanced, recurrent or metastatic endometrial cancer and we are hopeful that Zoptrex™ will provide clinicians and their patients with an effective therapy for treating the disease. We are indebted to all 512 patients who participated in this important clinical program and, hopefully, we will advance to providing a very important new therapy for this devastating cancer."

About the ZoptEC Pivotal Phase 3 Trial The ZoptEC pivotal Phase 3 trial was a fully-recruited (over 500 patients), open-label, randomized-controlled study, comparing the efficacy and safety of zoptarelin doxorubicin, a hybrid molecule composed of a synthetic peptide carrier and a well-known chemotherapy agent, doxorubicin, to doxorubicin alone. Patients were centrally randomized in a 1:1 ratio and received either Zoptrex™ (267 mg/m²) or doxorubicin (60 mg/m²) intravenously, every three weeks and for up to nine cycles. Response was evaluated every three cycles during treatment, and thereafter, every 12 weeks until progression. All patients were followed for survival as the primary efficacy endpoint ("EP"). Secondary EPs include progression-free survival, objective response-rate, and clinical benefit rate. The trial is being conducted under a Special Protocol Assessment with the U.S. Food and Drug Administration ("FDA").

For more information on this trial, please consult (ClinicalTrials.gov Identifier: NCT01767155; EudraCT No: 2012-005546-38; ZoptEC: Zoptarelin doxorubicin in endometrial cancer).

About Zoptarelin Doxorubicin Zoptrex™ (zoptarelin doxorubicin), a novel synthetic peptide carrier linked to doxorubicin as a New Chemical Entity (NCE), is the Company's lead oncology compound. Zoptrex™ is the first targeted oncological therapy using a peptide as the targeting agent and, therefore, it represents potentially a new tool in the treatment of cancer tumors that overexpress the LHRH receptor. The design of the compound allows for the specific binding and selective uptake of the cytotoxic conjugate by LHRH receptor-positive tumors, typically found in gynecological cancers, prostate cancer and some forms of breast cancer. Potential benefits of this targeted approach may include enhanced efficacy and a more favorable safety profile with lower incidence and severity of adverse events, as compared to doxorubicin. Based on the results of Phase 2 studies, the Company believes it may be efficacious for the treatment of ovarian and prostate cancer. If Zoptrex™ is approved as a therapy for endometrial cancer, the Company intends to develop it for these additional indications. The Company has licensed marketing rights to Zoptrex™ to Sinopharm A-Think for China, Hong Kong and Macau; to Orient EuroPharma for Taiwan and Southeast Asia; to Rafa Labs for Israel and the Palestinian territories and to Specialised Therapeutics for Australia and New Zealand.

About Endometrial Cancer Endometrial cancer is the most common gynecologic malignancy in developed countries and develops when abnormal cells amass to form a tumor in the lining of the uterus. It largely affects women over the age of 50 with a higher prevalence in Caucasians and a higher mortality rate among African Americans. According to the American Cancer Society, there will be approximately 50,000 new cases of endometrial cancer in the U.S. alone in 2015, with about 20% of recurring disease.

About Aeterna Zentaris Inc. Aeterna Zentaris is a specialty biopharmaceutical company engaged in developing and commercializing novel treatments in oncology, endocrinology and women's health. We are engaged in drug development activities and in the promotion of products for others. We recently concluded Phase 3 studies of two internally developed compounds. The focus of our business development efforts is the acquisition of licenses to products that

are relevant to our therapeutic areas of focus. We also intend to license out certain commercial rights of internally developed products to licensees in non-US territories where such out-licensing would enable us to ensure development, registration and launch of our product candidates. Our goal is to become a growth-oriented specialty biopharmaceutical company by pursuing successful development and commercialization of our product portfolio, achieving successful commercial presence and growth, while consistently delivering value to our shareholders, employees and the medical providers and patients who will benefit from our products. For more information, visit www.aezsinc.com.

Forward-Looking Statements This press release contains forward-looking statements made pursuant to the safe harbor provisions of the US Securities Litigation Reform Act of 1995. Forward-looking statements may include, but are not limited to statements preceded by, followed by, or that include the words “expects,” “believes,” “intends,” “anticipates,” and similar terms that relate to future events, performance, or our results. Forward-looking statements involve known and unknown risks and uncertainties that 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 R&D projects and clinical trials, the successful and timely completion of clinical studies, the risk that safety and efficacy data from any of our Phase 3 trials may not coincide with the data analyses from previously reported Phase 1 and/or Phase 2 clinical trials, the rejection or non-acceptance of any new drug application by one or more regulatory authorities and, more generally, uncertainties related to the regulatory process, the ability of the Company to efficiently commercialize one or more of its products or product candidates, 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 to protect our intellectual property, the potential of liability arising from shareholder lawsuits and general changes in economic conditions. Investors should consult the Company’s quarterly and annual filings with the Canadian and US securities commissions for additional information on risks and uncertainties relating to forward-looking statements. Investors are cautioned not to place undue reliance on these forward-looking statements. The Company does not undertake to update these forward-looking statements. We disclaim 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.