



Æterna Zentaris

Improving Life... Transforming Value

August, 2016

Forward Looking Statement

This presentation contains forward-looking statements made pursuant to the safe harbor provisions of the U.S. Securities Litigation Reform Act of 1995. Forward-looking statements involve known and unknown risks and uncertainties that could cause our 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 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 of the Company to protect its intellectual property 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 place undue reliance 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.

Near-term Value Drivers

Two Pivotal Phase III Trials Completion Expected by Year-end

- **Macrilen™ (macimorelin)** Oral ghrelin receptor agonist for assessing Adult Growth Hormone Deficiency (AGHD)
 - If approved, will be the only FDA-approved drug for assessing AGHD, representing a \$30M - \$50M annual market opportunity
 - Granted orphan drug status with approximately 40,000 AGHD tests conducted annually (US)
 - Significant market expansion opportunity for traumatic brain injury (TBI) patients at risk of developing AGHD
 - Pivotal Phase III trial completion expected Q3 2016, with top-line results by year-end 2016

- **Zoptrex™ (zoptarelin doxorubicin)** Targeted (LHRH receptor) cytotoxic drug for the treatment of advanced (stage III & IV) endometrial cancer (EC)
 - If approved, will be the first FDA-approved treatment for advanced EC, representing a \$300M - \$500M annual market opportunity (US)
 - Successful out-licensing for non-US territories: China, Hong Kong & Macau (Sinopharm A-Think); Taiwan & Southeast Asia (Orient EuroPharma); Israel & Palestine (Rafa Labs); further out-licensing discussions continue
 - Pivotal Phase III trial completion expected by year-end 2016, with top-line results expected Q1 2017

Foundation for Growth

Focused on Achieving Value Growth & Development

Commercial-stage promotion

- **Apifyn**® The only non-PSA based blood test for evaluating the risk of prostate cancer; exclusive US promotion agreement with Armune BioSciences Inc.
- **Saizen**® [somatropin (rDNA origin) for Injection] Growth hormone replacement therapy for children and adults, co-promoted with EMD Serono in the US

Portfolio Growth

- Actively pursuing additional portfolio opportunities via product in-license/acquisition
- Innovative technology platform supporting long-term growth (“targeted cytotoxic therapy”)

Proven Successful Leadership

- Proven leadership with record of creating significant incremental shareholder value

Financial Overview

- **Cash on hand as of June 30, 2016** → \$26.2 million
- **Planned 2016 average operating cash used in operations** → between \$2.5 and \$2.7 million per month
- **Outstanding shares as of August 9, 2016** → 9.9 million shares
- **No debt**
- **Traded on NASDAQ (“AEZS”) and TSX (“AEZ”)**

Strategic Growth Plan

- Pursue successful registration & commercialization of Macrilen™ in endocrinology and Zoptrex™ in oncology in the U.S.
- Out-license rights to Macrilen™ and Zoptrex™ outside the U.S.
- Achieve successful commercial presence and growth
 - Commercialization of Apifyny®, Saizen® & additional products via in-license/acquisition
- Develop Zoptrex™ for additional indications and develop follow-on oncology compound
- Become a profitable, growth oriented specialty biopharmaceutical company by year-end 2018



Proven Leadership

David Dodd; President & CEO

- Successful experience in achieving significant business growth and multi-billion dollar value-transformations in Pharma & Biotech (U.S. & Global)
 - Big pharma (BMS, Divisional GM; Wyeth, US Pharma Head)
 - Mid pharma (Solvay, CEO)
 - Biotech (Serologicals, public company CEO; BioReliance, private equity buy-out/restructuring Chairman/CEO; Stem Cell Sciences, public company Executive Chairman; GeoVax, public company Chairman)

Richard Sachse, M.D., Ph.D.; Chief Scientific Officer & Chief Medical Officer

- Successfully led and contributed to development and commercialization of therapeutic products for Global pharmaceutical companies
 - Head of Global Translational Medicine, Boehringer Ingelheim
 - Project and Program Leadership at Schwarz Pharma/UCB
 - Project Leadership at Bayer AG

Jude Dinges; Chief Commercial Officer

- Product, sales and marketing leadership at Merck, Novartis and Amgen
- Therapeutic experience includes: Anti-virals, Cardiovascular, Endocrinology, Neurology, Osteoporosis, Vaccines
- Direct product commercialization experience includes: Crixivan[®], Exelon[®] Patch, Fosomax[®], Maxalt[®], PedvaxHIB[®], Prolia[®], Proscar[®], Singulair[®], Tekturna[®], Vioxx[®], Xolair[®], Zetia[®]

Pipeline – Supporting Long-Term Growth

Product Candidate	Discovery	Preclinical	Phase 1	Phase 2	Phase 3
Zoptrex™ (zoptarelin doxorubicin)				Endometrial Cancer	
Macrilen™ (macimorelin)					AGHD
Zoptrex™ (zoptarelin doxorubicin)				Ovarian ⁽¹⁾ Cancer	
Zoptrex™ (zoptarelin doxorubicin)			Prostate ⁽²⁾ Cancer		
AEZS-120		Prostate Cancer ⁽³⁾			
Erk inhibitors	Oncology ⁽⁴⁾				
LHRH – Disorazol Z	Oncology				
Compound Library – MUSC ⁽⁵⁾					

(1) Phase 2 in ovarian cancer completed.

(2) Investigator-driven and sponsored Phase 2 trial in castration and taxane resistant prostate cancer completed.

(3) Potential oral prostate cancer vaccine available for co-development/out-licensing.

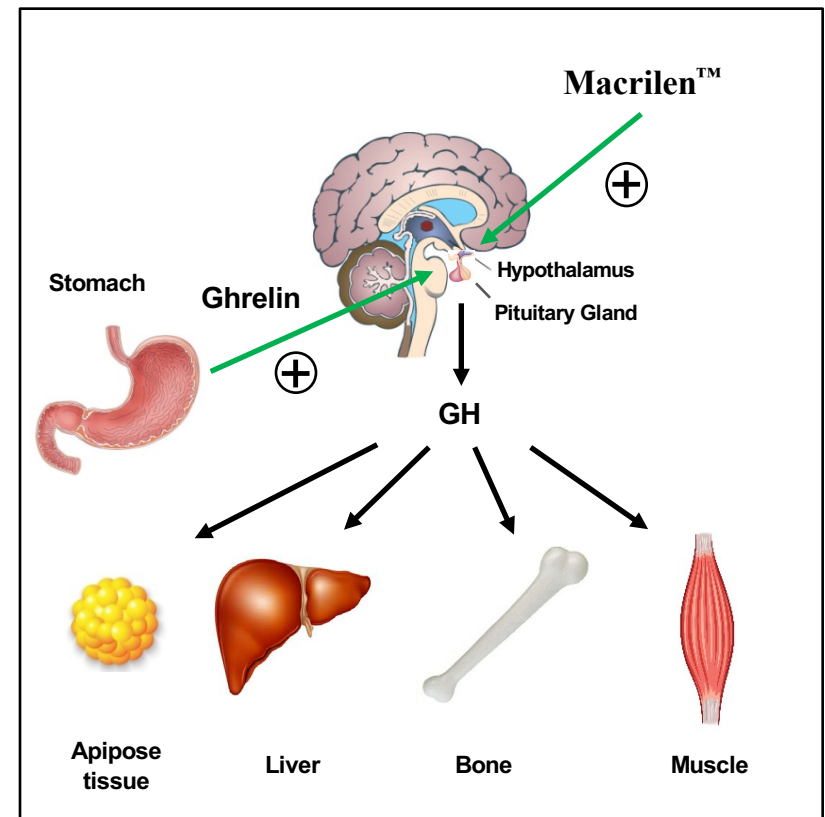
(4) Available for co-development/out-licensing.

(5) Compound library transferred to MUSC (Medical University of South Carolina); Aeterna Zentaris has access to future potential development candidates.

Macrilen™ (macimorelin)

Macrilen™ -- A potent stimulator of GH release

- Growth hormone (GH) is a potent regulator of lipid, sugar and protein metabolism
- GH secretion is regulated by neuro-endocrine hormones released by the hypothalamus (e.g. GHRH), various physiologic stimuli incl. exercise and sleep, as well as Ghrelin
- Ghrelin acts as a growth hormone secretagogue (GHS) and potently stimulates GH release from the pituitary
- **Macrilen™** is readily absorbed from the gastrointestinal tract – acts in a similar way as ghrelin (GH secretion)



Macrilen™

Product

- Novel orally-active ghrelin receptor agonist that induces a fasting patient's Growth Hormone (GH) secretion

If FDA-Approved...A Strong Value Proposition

- **Orphan-drug status** granted by FDA
- **Novel:** the **only FDA approved** product for assessing AGHD
- **Accurate:** comparable to current standard procedures
- **Safe:** well tolerated
- **Convenient vs “Gold Standard” Insulin Tolerance Test (“ITT”):**
 - Oral vs IV administration
 - Simple: single blood draw possible vs 3-4 hour procedure for ITT
 - No induced hypoglycemia resulting in less burden and safety issues for patients
 - Less medical supervision



Assessing Adult Growth Hormone Deficiency

Insulin Tolerance Test (“ITT”)



Fasted Patient



Initial Blood Draw

Physician Supervision

2 Hours

Hypoglycemia induced via
Insulin IV administration



Multiple blood draws and monitoring
of blood glucose levels



Up to several Hours

Continued medical
supervision

Macrilen™



Fasted Patient



Initial Blood Draw

No Special Medical Supervision

1 Hour



1-2 blood draws



Patient Drinks
Solution

No patient
restrictions
or
necessary care

Significant Market Expansion Opportunity

Assessing Traumatic Brain Injury (TBI) Victims

- Estimated 215,000 adult patients (≥ 19 years) annually hospitalized for Traumatic Brain Injury (TBI) in U.S.*
- An estimated 40,850 (19%) of severe and moderate hospitalized TBI victims are at risk to develop GHD (Growth Hormone Deficiency)**
- Moderate & severe TBI patients should be assessed for GHD at 3, 6 and 12 months post TBI-event, for a potential total of 122,000 annual evaluations in the U.S. alone***

TBI evaluation would fall into our indication!

* Source: Centers for Disease Control and Prevention (CDC) – MMWR, 2010 and 2011

** Source: Agha et al., British Journal of Neurosurgery, 2007

** Source: Fernandez-Rodrigues et al, Frontiers in Endocrinology, 2011

***Source: Popovic et al. AGHD, Frontiers of Hormone Research, Basel, Karger, 2005

Estimated Annual US Market Potential for GHD Assessment

GHD: Growth Hormone Deficiency

	Tests
Adult*	36,000
Traumatic Brain Injury (adult)**	122,000
Pediatric*	41,000
Traumatic Brain Injury (pediatric)**	34,000

Total GHD tests: ~ 233,000

* Source: Navigant 2009

** Source: Centers for Disease Control and Prevention (CDC) – MMWR, 2010 and 2011

** Source: Agha et al., British Journal of Neurosurgery, 2007

** Source: Fernandez-Rodriguez et al, Frontiers in Endocrinology, 2011

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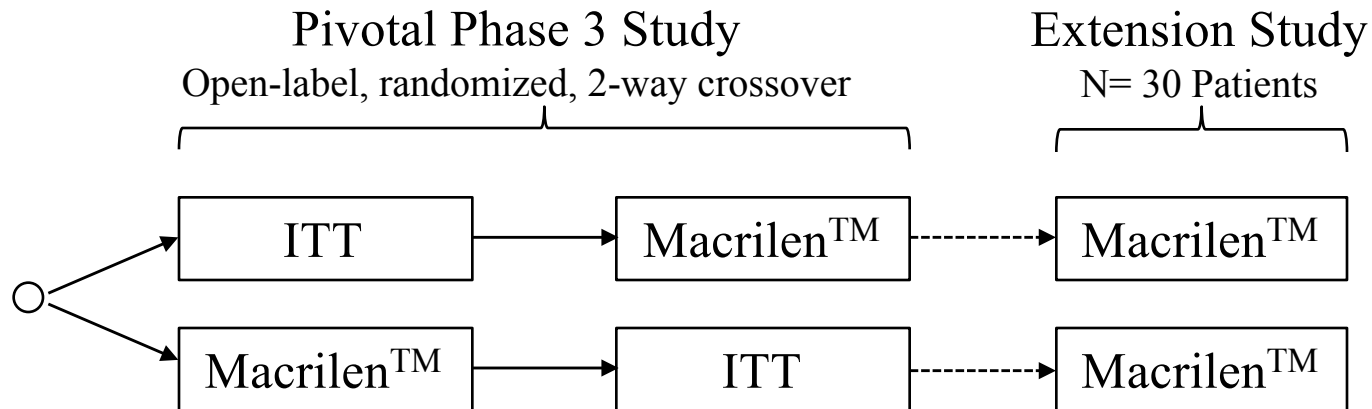
Macrilen™ Commercial Considerations

- Initial Target Market: ~40,000 Adult Growth Hormone Deficiency (AGHD) tests conducted in the U.S. annually.
- If approved, Macrilen™ will be the only available FDA-approved product to assess AGHD.
- First year estimated AGHD confirmatory market penetration targeted for ~ 40-50%, growing to ~85% by year three.
- Macrilen™ adoption will be driven by ~ 2,500 ENDOs, enabling complete coverage by small specialty sales force.
- Significant market expansion opportunity for Macrilen™ in traumatic brain injury (TBI) patients.

Macrilen™ Access, Reimbursement & Pricing

- Market Access at Launch
 - ENDOs and patient demand will drive payer access
 - May require contracting with select GPOs and or MCOs
- Anticipated Payer Coverage and Reimbursement
 - Seeking coverage on both pharmacy and medical benefit (required ‘j-code’)
 - Purchase and distribution through specialty pharmacy, institutional and hospital markets
- Pricing
 - Will consider economics of costs related to current standard ITT; orphan drug designation; if approved, the only FDA-approved product for this indication

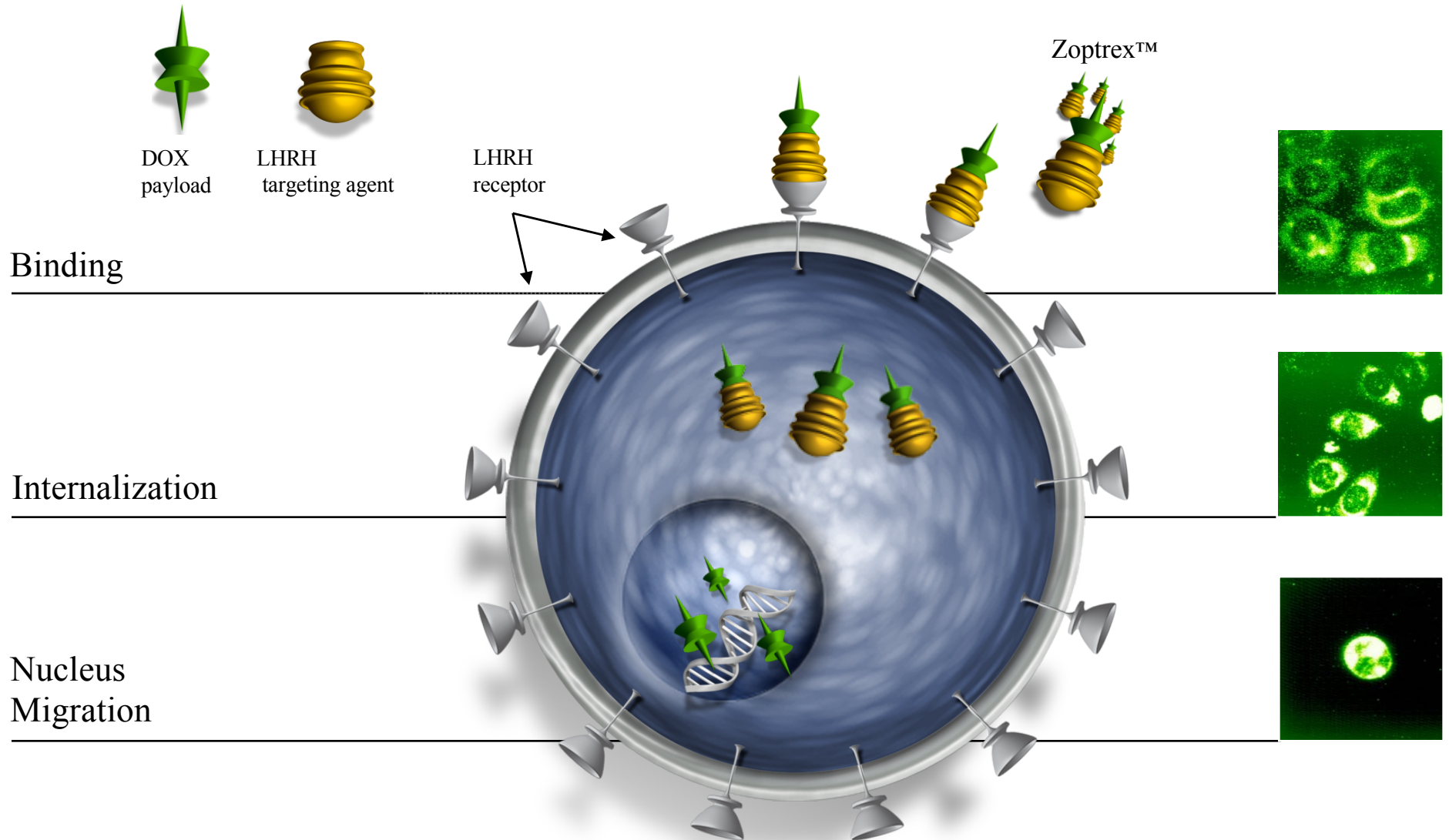
Macrilen™ -- Confirmatory Phase 3 Trial Design



- **Primary Objective**
 - Validation of macimorelin for the diagnosis of AGHD, using the ITT as comparator
- **Co-Primary Efficacy Variables**
 - “Percent Negative Agreement” and “Percent Positive Agreement”
- **Patients**
 - Patients with suspected GHD (low ↔ high risk) and 25 healthy subjects
 - N = at least 110 (≥ 55 with positive and ≥ 55 with negative ITT outcome)

Zoptrex™ (zoptarelin doxorubicin)

Zoptrex™ – Targeted Cytotoxic Therapy



Ref.: Westphalen et al. Int J Oncol. 2000

A Potential Breakthrough in Cancer Therapy

Zoptrex™

New Chemical Entity (NCE) composed of a targeted synthetic peptide carrier linked to doxorubicin

If FDA-Approved...A Strong Value Proposition

Improved, targeted delivery of doxorubicin with improved benefit-risk profile
First FDA-approved medical therapy for treating advanced, recurrent endometrial cancer (Stage III & IV)

Status

- Phase 3 trial in Stage III & IV endometrial cancer – Expected completion by year-end, 2016; conducted under a Special Protocol Assessment (SPA) with the FDA
 - Fully enrolled (over 500 patients) / 125 active sites in North America, Europe and Israel
 - Final Interim Results: DSMB recommendation to continue ZoptEC trial to completion (October 2015)

Zoptrex™ – Multiple Potential Applications

Tumor Site	Estimated New US Cases 2016	Clinical Evaluation
Endometrium	60,050	
Locally advanced, recurrent or metastatic, failure after platinum-taxane	10,000	Phase 3; ZoptEC trial; NCT01767155 (n=500), zoptarelin dox vs doxorubicin
Ovary	22,280	
Platinum refractory or resistant	14,240	Phase 2; NCT00569257 (n=42)
Prostate	180,890	
Castration and taxane resistant	26,120	Phase 2; NCT01240629 (n=25)
Breast	246,660	
Chemotherapy refractory triple negative breast cancer	40,450	Phase 2; NCT01698281 (n=8) On-hold; restart following ZoptEC trial
Bladder	76,960	
Locally advanced unresectable or metastatic	16,390	Phase 1; NCT01234519 (n=13) On-hold; restart following ZoptEC trial

Source: Cancer Facts & Figures 2016, ACS; literature estimates

Compelling Phase 2 Results

Tumor Site (Study)	Median Overall Survival Zoptrex™	Median Overall Survival (Literature data) Doxorubicin	Cardiotoxicity Zoptrex™	Cardiotoxicity (Literature data) Doxorubicin
Endometrium (AGO-GYN5)	14.9 months		0/44 reports	
Endometrium (Thigpen <i>et al.</i> (2004), J Clin Oncol 22:3902-3908)		9.2 months		7/150 Grade 1 15/150 Grade 2 4/150 Grade 3 2/150 Grade 4
Endometrium (Aapro <i>et al.</i> (2003), Annals of Oncology 14:441-448)		7.0 months		1/87 Grade 1 1/87 Grade 3
Ovary (AGO-GYN5)	12.2 months		0/42 reports	

AGO-GYN5:

Stratum B : Advanced or recurrent endometrial cancer expressing LHRH receptors

Stratum A : Platinum resistant ovarian cancer expressing LHRH receptors

Zoptrex™ Commercial Plans in the US

■ Target Medical Oncologists

- Universe includes 12,500 total
- 2,000 gynecological oncologists

■ Oncology Specialty Sales Force

- Segmented strategy focused on high value targets, key commercial insurers and major medical centers
- Zoptrex™ alone: ~30-50 sales representatives
- Zoptrex™ + in-license brand: ~50-75 sales representatives

Zoptrex™ Commercial Considerations

- U.S. annual incidence ~10,000 women treated for advanced endometrial cancer
- Final approved label and accompanying efficacy & safety data of Zoptrex™ may yield optimal utilization and adoption
 - Overall survival (OS) outcome in patients treated with Zoptrex™ will be critical to acceptance
 - Reduced patient complications and associated cost reductions with improved safety profile vs doxorubicin
- Medical Oncologists are data-driven clinicians who seek evidence from clinical trials to guide and validate their treatment decisions
- Given no FDA-approved drugs and no consensus standards for the treatment of late stage, locally advanced, recurrent or metastatic endometrial cancer, we believe a successful trial resulting in FDA-approval of Zoptrex™ should yield **rapid and significant utilization upon commercial launch**

Zoptrex™ Access, Reimbursement & Pricing

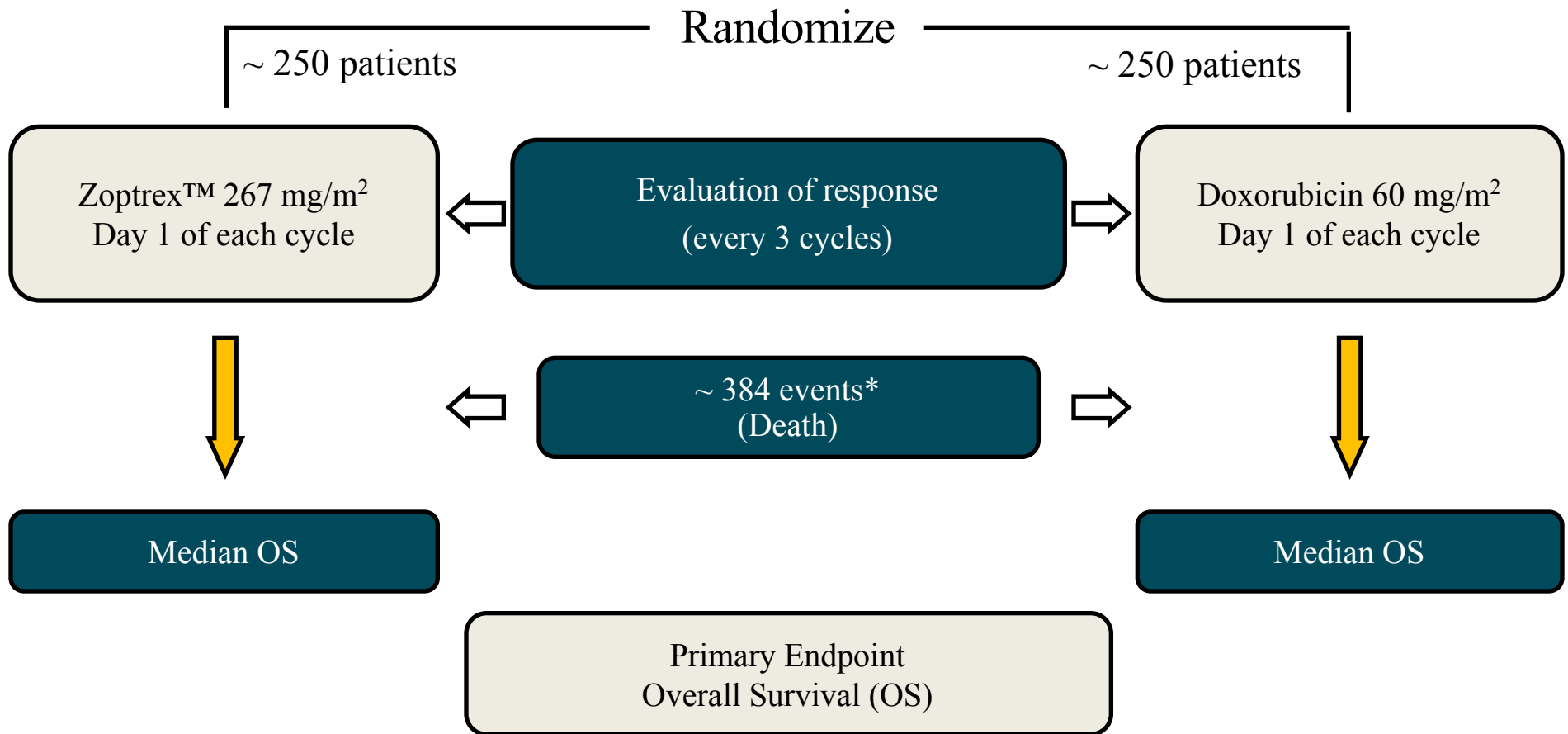
- Significant Market Access at Launch if Zoptrex™ Successful
 - Competing therapies lack definitive results and guideline consensus
 - Zoptrex™ expected to be available to all appropriate patients consistent with approved label
- Anticipated Payer Coverage and Reimbursement
 - Initial launch with a ‘temporary’ reimbursement code, followed by a permanent CMS-established ‘J code’ within 18 months
 - Traditional “buy and bill” therapy
- Potential for Optimal Pricing & Utilization
 - First and only FDA-approved treatment for advanced endometrial cancer
 - Final label will impact price and utilization

Significant Market Potential

Estimated US Annual Market Potential Treating Advanced, Recurrent Cancers (Stage III & IV)

Type of Cancer	Annual Market Potential
Endometrial Cancer	\$300 million -- \$500 million
Other Cancers (Ovarian; Prostate; etc.)	> \$500 million
Total	~ \$1 billion

Zoptrex™ Pivotal Phase 3 Study Under SPA



Each cycle = 21 days

- Pre-planned interim analyses completed at ~128 and ~192 events
- SPA: Special Protocol Assessment agreement with the FDA

Zoptrex™ ... Next Steps

Global Commercial Development Plan

- Prepare for registration and commercial launch in the US
- Establish partnerships in non-strategic territories
 - Out-licensing agreements already established with
 - **Sinopharm A-Think** for China, Hong Kong and Macau;
 - **Orient EuroPharma** for Taiwan and Southeast Asia;
 - **Rafa Labs** for Israel and Palestine
 - Other out-licensing agreements potentially to come



Zoptrex™ ... Life Cycle Management

- **Strengthen patent protection**
 - Process patent filed worldwide
 - Expected reduction of manufacturing costs >50%
- **Following successful development in endometrial cancer therapy...**
 - Ovarian cancer therapy (Phase 2 trial successfully completed)
 - Prostate cancer therapy (Investigator Initiated Phase 2 trial successfully completed)
 - Additional indications with clinical data (bladder, breast cancer)
 - LHRH-Disorazol Z (new patent protected optimized follow-up molecule)



Portfolio Development

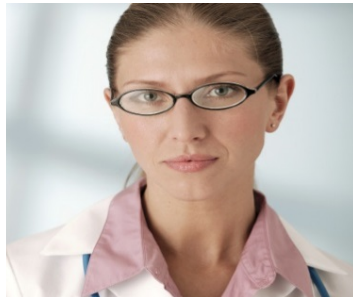
Portfolio Development

- Aeterna Zentaris is seeking to in-license/acquire currently marketed products for the US market
 - Will also consider products currently in registration
- Therapeutic Areas of Interest Include
 - Oncology
 - Endocrinology
 - Urology
 - Woman's Health
- Will favorably consider deals re ex-US rights for Zoptrex™ and/or Macrilen™ which include reciprocal rights to US marketed product(s)

Vision

Transform Aeterna Zentaris from development stage to a profitable, commercially operating specialty biopharmaceutical company through:

- Working towards successful development and commercialization of our near-term value drivers
- Pursuing further portfolio additions



Investment Opportunity

Major Near-term Value-Driver Events: Two Pivotal Phase III Trials Completion Expected by Year-end

- **Macrilen™ (macimorelin)** Oral ghrelin receptor agonist for assessing Adult Growth Hormone Deficiency (AGHD)
- **Zoptrex™ (zoptarelin doxorubicin)** Targeted (LHRH receptor) cytotoxic drug for the treatment of advanced (stage III & IV) endometrial cancer (EC)

Commercial-stage promotion

- **Apify®** The only non-PSA based blood test for evaluating the risk of prostate cancer; exclusive US promotion agreement with Armune BioSciences Inc.
- **Saizen®** [somatropin (rDNA origin) for Injection] Growth hormone replacement therapy for children and adults, co-promoted with EMD Serono in the US

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