

# Aeterna Zentaris

Investor Presentation

May 18, 2011

Aeterna Zentaris

*Committed to cure*

# Forward-Looking Statements

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, which could cause Æterna Zentaris' 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, the successful and timely completion of clinical studies, the ability of the Company to take advantage of business opportunities in the pharmaceutical industry, 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 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 we are required to do so by a governmental authority or under applicable law.

# Company Overview

- **Late-stage drug development company primarily focused on potential treatments for various cancers**
  - Perifosine: 2 pivotal Phase 3 studies ongoing
  - AEZS-108: completed positive Phase 2 for gynecological cancers
  - AEZS-130: currently in Phase 3 under a Special Protocol Assessment as oral diagnostic for AGHD
- **AEZS-130: NDA filing planned in 2011**
- **Perifosine: NDA and MAA filings planned in 2012**
- **Solid cash position sufficient to take us to these filings**

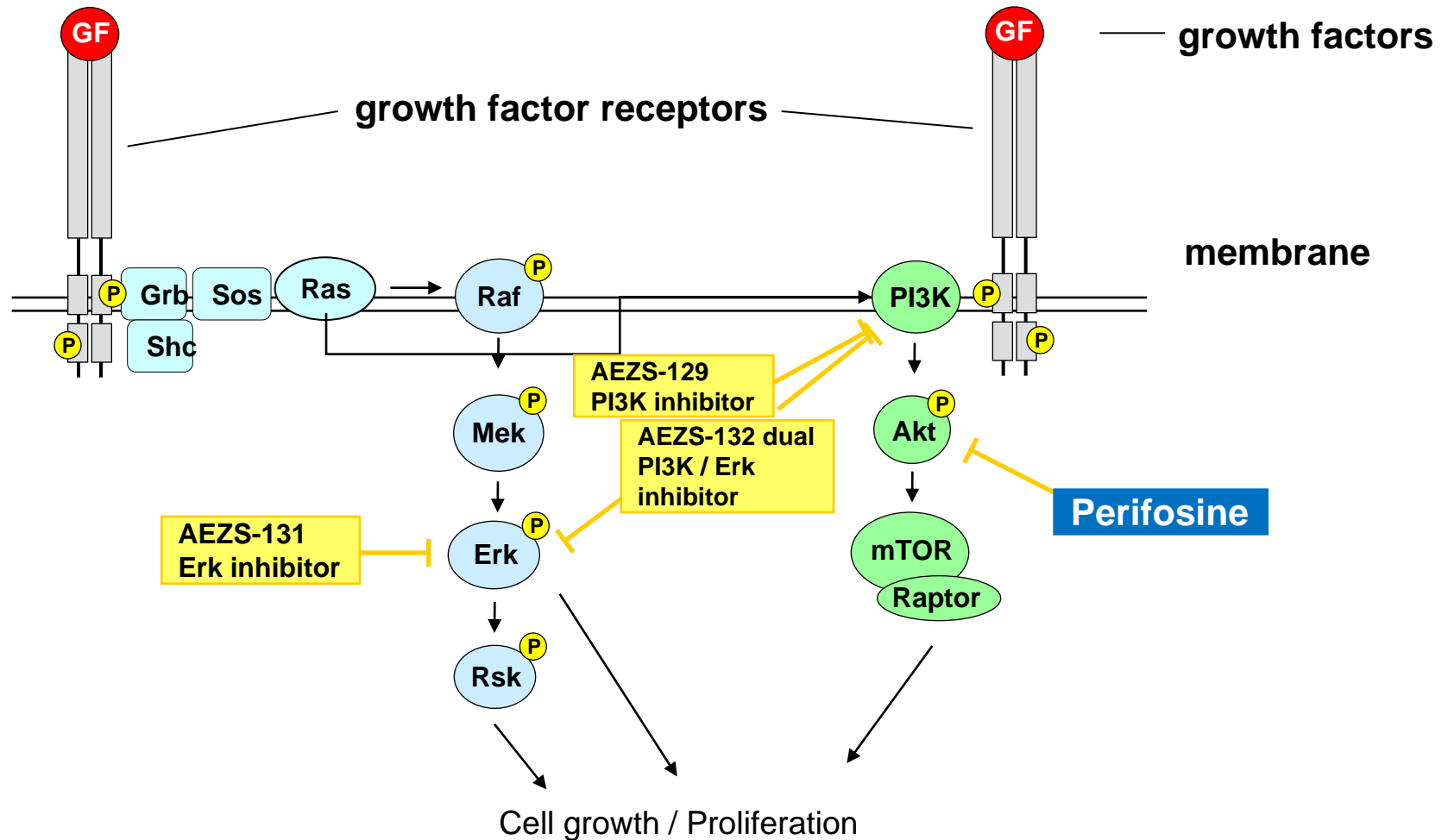
# Management Team

<b>Juergen Ernst</b> Chairman	30+ years experience: Solvay
<b>Juergen Engel, Ph.D.</b> President and CEO	30+ years experience: ASTA Medica
<b>Paul Blake, M.D.</b> Senior VP and CMO	25+ years experience: Cephalon, SmithKline Beecham (now GSK), ICI Pharmaceuticals (now Astra Zeneca)
<b>Nicholas J. Pelliccione, Ph.D.</b> Senior VP, Regulatory Affairs and QA	20+ years experience: Chugai Pharma USA , Schering-Plough
<b>Matthias Seeber, M.B.A.</b> Senior VP, Administration and Legal Affairs	15+ years experience: Altana AG, Deka Investment Management, Dresdner Bank AG
<b>Dennis Turpin, CA</b> Senior VP and CFO	20+ years experience: Coopers & Lybrand (now PWC)

# Pipeline

Compound	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Commercial
<b>Perifosine</b>	Advanced Colorectal Cancer	Partners: Keryx (North America) & Handok (Korea) & Yakult (Japan)				
	Multiple Myeloma	Partners: Keryx (North America) & Handok (Korea) & Yakult (Japan)				
	Kidney Cancer and Others	Partners: Keryx (North America) & Handok (Korea) & Yakult (Japan)				
<b>AEZS-108</b>	Endometrial Cancer					
	Ovarian Cancer					
	Refractory Prostate Cancer					
	Bladder Cancer					
	Other Cancers					
<b>AEZS-112</b>	Solid Tumor					
<b>AEZS-120</b> Tumor Vaccine	Prostate Cancer					
<b>AEZS-129/131/132</b> PI3K/Erk Inhibitor	Oncology					
<b>Cetrotide®</b>	<i>In Vitro</i> Fertilization	Partners: Merck Serono, Nippon Kayaku / Shionogi (Japan)				
<b>AEZS-130</b>	Diagnostic – Adult Growth Hormone Deficiency					
<b>AEZS-130</b> Ghrelin Agonist	Cancer Cachexia and Other Therapeutic Use					
<b>AEZS-123</b> Ghrelin Antagonist	Endocrinology					

# AEZS Inhibitor Programs



# Perifosine Overview

## More than 2,000 patients in Phase 1 and 2

- Single agent and in combination (oral dosage 50-100 mg per day)
- Well tolerated at current doses

## Indications

- Refractory Advanced Colorectal Cancer (CRC): Phase 3
  - SPA and Fast-Track review granted by FDA
  - Positive Scientific Advice granted by EMA
- Multiple Myeloma (MM): Phase 3
  - SPA, orphan-drug designation and Fast-Track review granted by FDA
  - Orphan Drug Designation and Positive Scientific Advice granted by EMA
- Phase 1/2 studies ongoing in other tumor types

# Perifosine Overview

## Potential market from Decision Resources

- Refractory Advanced Colorectal Cancer (CRC)
  - One of the most common cancer worldwide
  - 480,000 newly diagnosed cases in 2010 for the seven major-markets
  - In U.S., 160,000 diagnosed cases with 28,000 in stage IV
- Multiple Myeloma (MM)
  - The second most prevalent blood cancer
  - 60,000 death per year
  - 130,000 diagnosed prevalent cases expected in 2010 for the seven major-markets
  - In U.S., 55,000 diagnosed prevalent cases expected in 2010
  - Treatable pool for perifosine third line or more nearly 25,000 patients in the U.S.
  - The relapsed/refractory patient populations in 2010 will be in total 32,000 cases, with 14,000 cases for the U.S.





# Perifosine in Colorectal Cancer

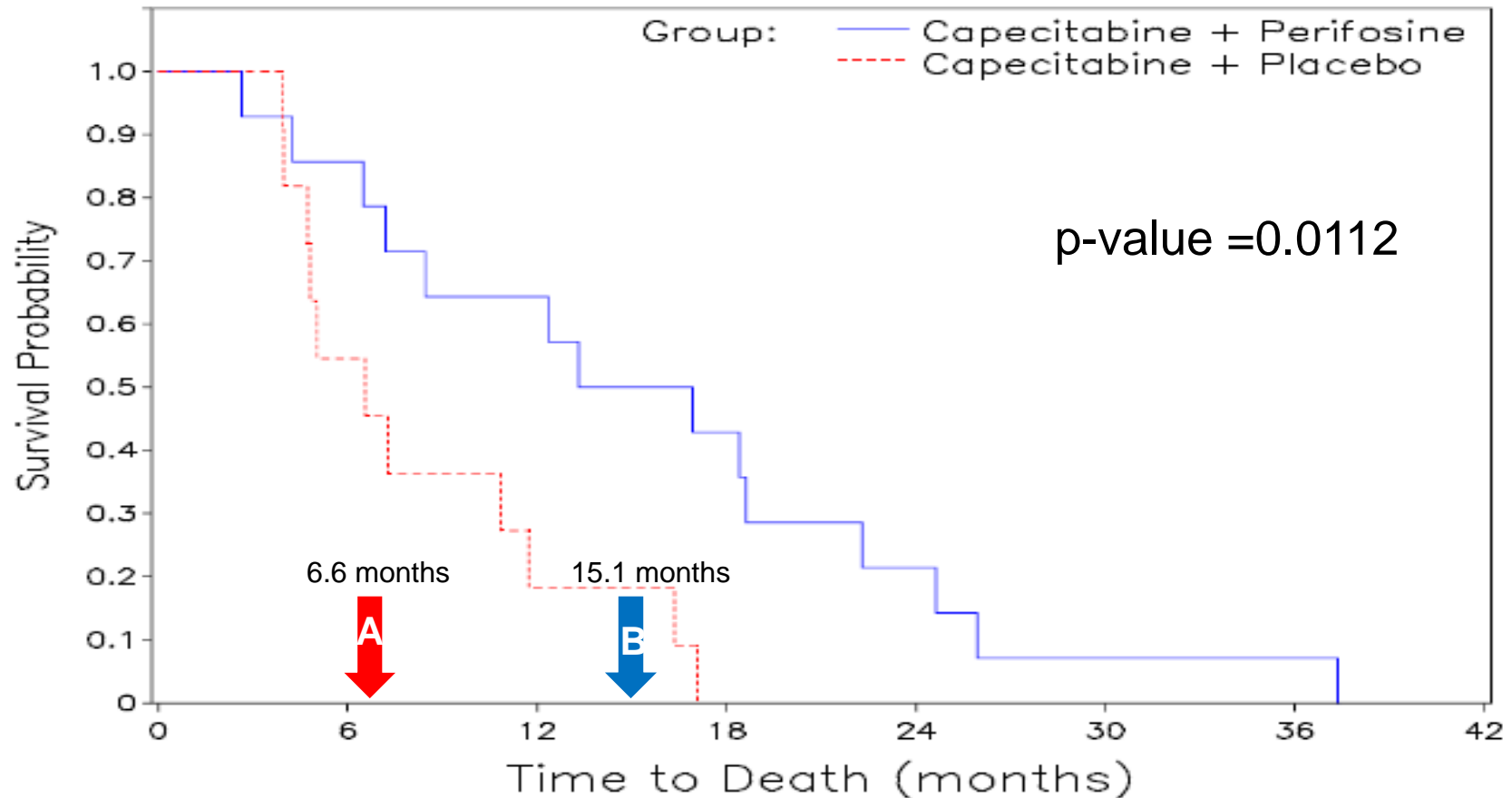
## Phase 2 Results in Advanced Metastatic CRC – Responses

Group	Number (n)	CR n (%)	PR n (%)	CBR n (%)	Median TTP	OS
Xeloda® + perifosine	20	1 (5%)	3 (15%)	15 (75%)	28 weeks	17.7 months
Xeloda® + placebo	15	0	1 (7%)	6 (40%)	11 weeks	10.9 months
CR – Complete response; PR – Partial response; CBR – Clinical benefit response; TTP– Time to progression					p = 0.0012	p = 0.0161

- Treatment regimen: Xeloda® 825 mg/m<sup>2</sup> BID d1-14; perifosine 50 mg daily
- Duration of responses:
  - Xeloda® + perifosine: CR – 36 months; PR – 21, 19, 11 months
  - Xeloda® + placebo: PR – 7 months

Ref.: D. Richards et al. ASCO 2010

## Phase 2 Results in Advanced Metastatic CRC – Subgroup of 5-FU Refractory Patients: Median Overall Survival



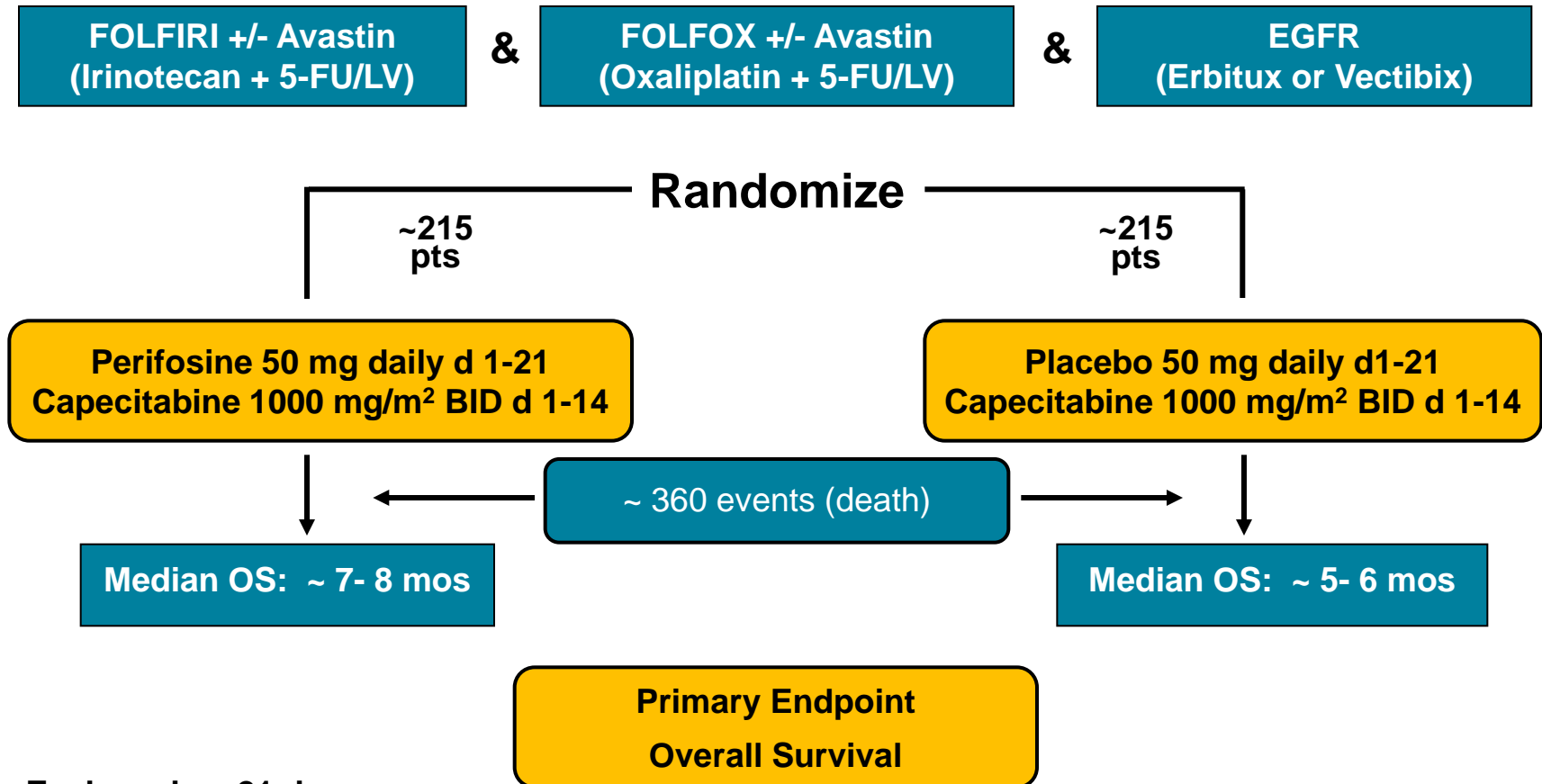
**A = 11 patients [95% CI (4.7, 11.7)]**

**B = 14 patients [95% CI (7.3, 22.3)]**

Ref.: D. Richards *et al.* ASCO 2010

# Phase 3 X-PECT Study in Refractory Advanced CRC – SPA with FDA and Positive Scientific Advice by EMA

## Prior Treatment with (3<sup>rd</sup> Line or >)





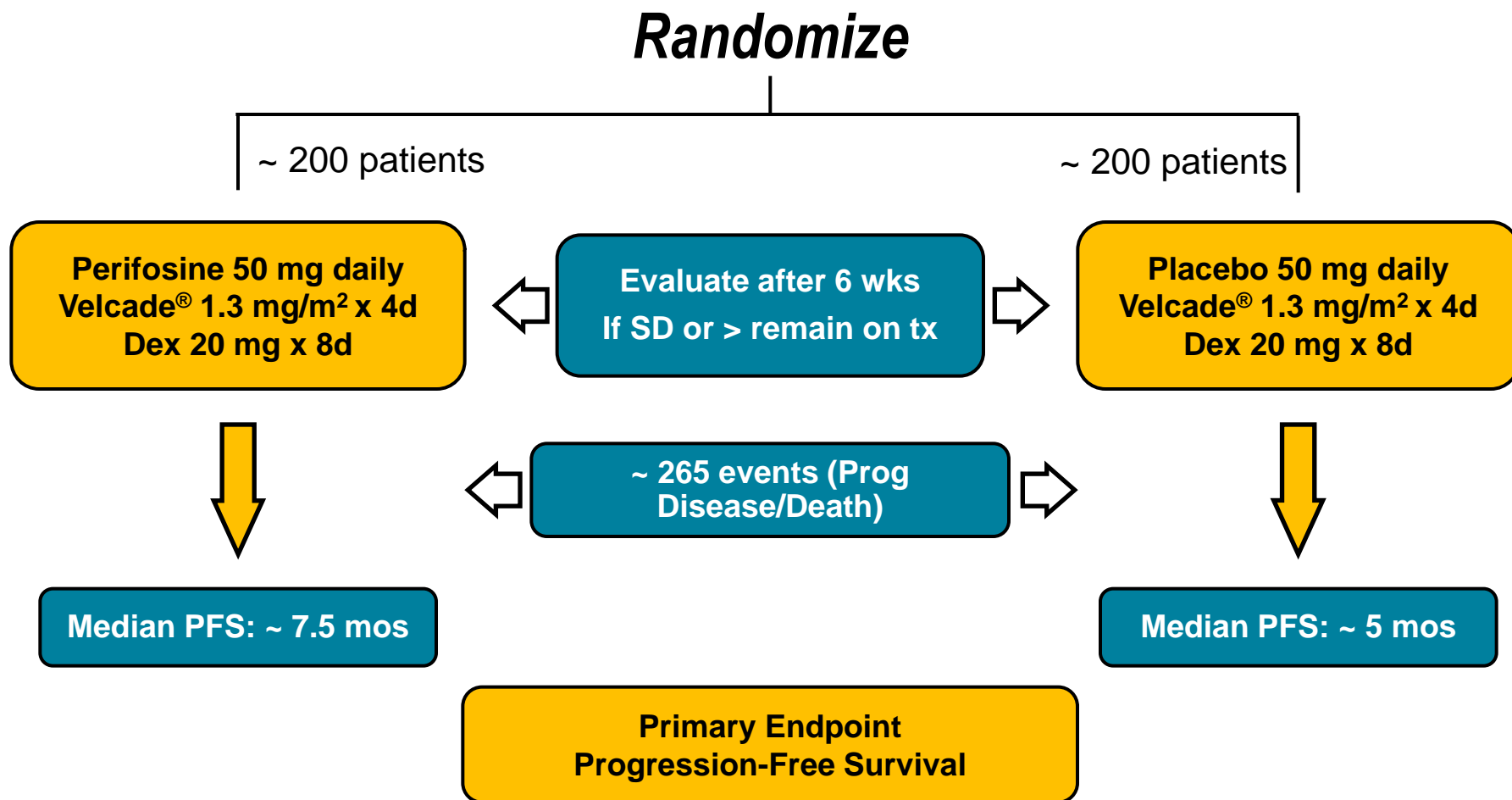
# Perifosine in Multiple Myeloma

# Phase 1/2 Results in MM

**Perifosine + Velcade® +/- Dexamethasone show encouraging median progression-free survival (PFS) and overall response rate (ORR) in Velcade relapsed/refractory patients**

	<b>Patients (n)</b>	<b>ORR</b>	<b>Median PFS</b>
<b>Velcade® refractory</b>	<b>53</b>	<b>32%</b>	<b>5.7 months</b>
<b>Velcade® relapsed</b>	<b>20</b>	<b>65%</b>	<b>8.8 months</b>
<b>All patients</b>	<b>73</b>	<b>41%</b>	<b>6.4 months</b>

# Phase 3 Study in MM – SPA with FDA and Positive Scientific Advice by EMA



Each cycle = 21 days

# Phase 1/2 Results: Perifosine + Revlimid + Dex in Multiple Myeloma

Best response (N = 30 pts)	N (%)	Duration on Tx (mos) Median (range)	≥ PR	≥ MR
Near Complete Response (nCR)	4 (13%)	32+, 32+, 28, 6	50%	73%
Very Good Partial Response (VGPR)	3 (10%)	35, 7, 4		
Partial Response (PR)	8 (27%)	Median 5.5 (4 – 29)		
Minimal Reponse (MR)	7 (23%)	Median 12 (2 – 34)		
Stable Disease (SD)	6 (20%)	Median 3 (2 – 30)		
Progression (PD)	2 (7%)	9, 4 weeks		

- Median Follow-up: 27 months, Median PFS 10.8 months
- Median OS 30.6 months
- Peri Rev Dex was well tolerated and demonstrated encouraging clinical activity and overall survival
- Seven patients remain progression-free and 15/30 patients remain alive as of Nov. 2010
- Pharmacodynamic studies correlating pAkt levels and response are being finalized

Ref.: Jakubowiak et al. ASH 2010





# Perifosine in Lymphomas

## Patients responding to perifosine alone

UPN	Age	Dx	Mol. Cyto-genetics	Prior Tx	Tx Duration (mos)	Response (mos)
3	38	CLL	del(17p13)	Auto-SCT, Allo-SCT, DLI, Campath	15	12
18	70	CLL	Normal	R-FC, Campath, R-Benda	9	8
22	61	CLL	del(17p13)	Fluda, HDS and Auto-SCT	10+	9+
26	63	CLL	del(17p13)	FMCD/DHAP, F-FC, Campath, R-Benda	5	4

## Conclusions

- Peri/Sor therapy had a good toxicity profile and was well tolerated (26 enrolled pts)
- Combination therapy has significant anti-lymphoma activity in relapsed/refractory HL
- Perifosine alone induced prolonged responses in high-risk, heavily pretreated CLL pts

*Ref.: Carlo-Stella et al., ASH 2010*

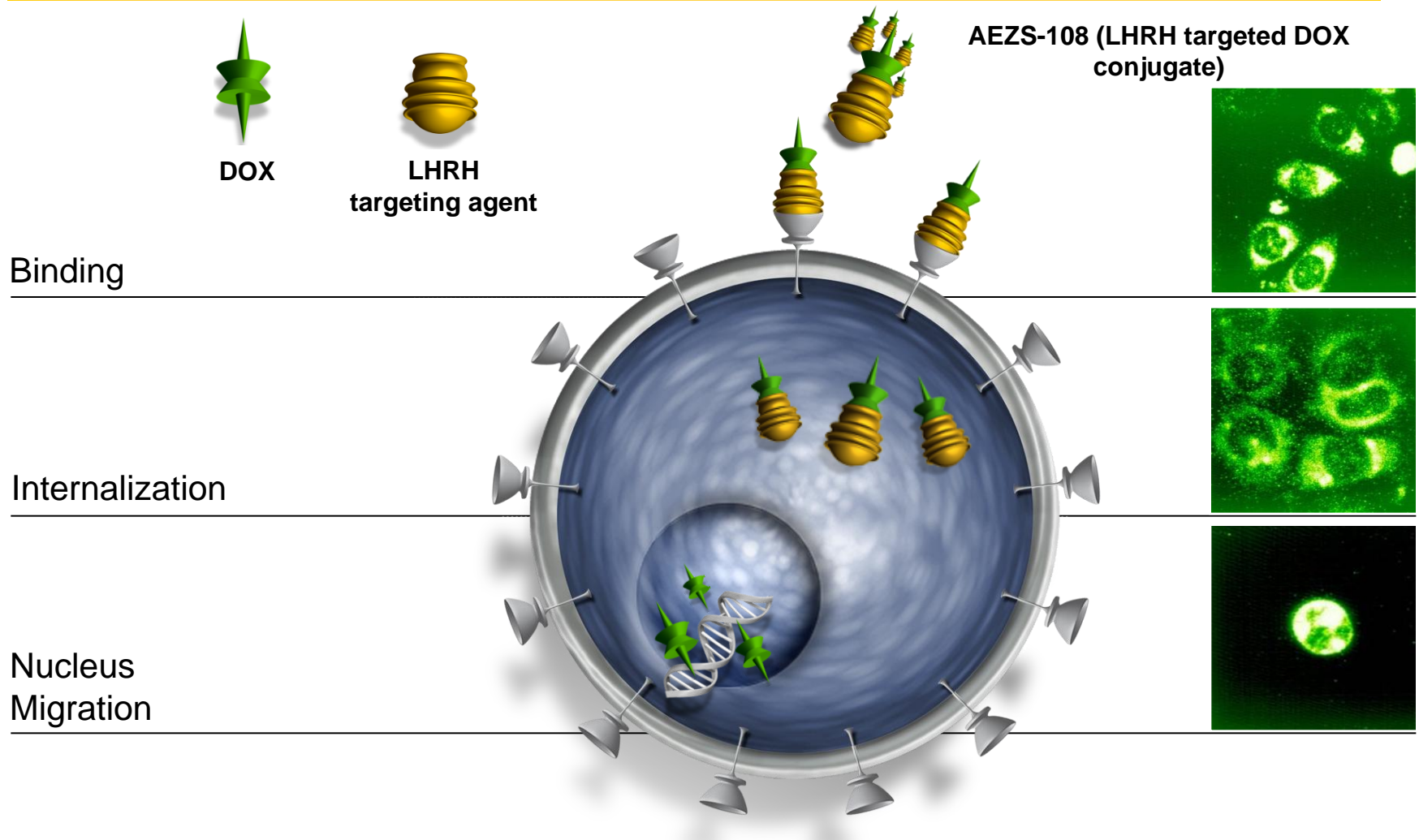


# AEZS-108

## LHRH receptor

- Ideal target for personalized medicine approach
  - Expressed in human cancer tissue (e.g. breast, endometrial, ovarian, prostate and bladder cancer)
  - Only detectable in reproductive tissue and pituitary
  - LHRH agonist can be used for targeting LHRH receptors
  - Companion diagnostic test can be developed

# AEZS-108 – Trojan Horse for Cancer Cells



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

# AEZS-108 – Phase 2 Study in Endometrial and Ovarian Cancer

- Open-label study in patients with advanced or recurrent endometrial and platinum-resistant ovarian cancer
  - Study performed by German Oncology Group (AGO)
  - Two-stage design (Simon Design) with up to 82 patients, 41 patients for each indication
- Status
  - Response criteria for opening stage 2 were met for both indications
  - Preliminary evaluation revealed that primary endpoint of 5 or more responders was met for both indications
  - Positive ovarian cancer results presented at ASCO 2010
  - Positive endometrial cancer results presented at EORTC-NCI-AACR (ENA) 2010

# AEZS-108 Phase 2 Study Refractory Ovarian Cancer

Number (n)	PR n (%)	SD n (%)	CBR n (%)	TTP (months)	OS (months)
42	5 (11.9%)	11 (26.2%)	16 (38%)	3.5	15.6

- Treatment regimen: 267 mg/m<sup>2</sup> IV infusion every 3 weeks
- Toxicity
  - No cardiotoxicity was observed
  - Hematological toxicity was rapidly reversible
  - OS for Topotecan and Doxil: 8-9 months

Ref.: G. Emons et al. ASCO 2010

# AEZS-108 Phase 2 Study

## Recurrent Endometrial Cancer

Number (n)	CR n (%)	PR n (%)	SD n (%)	CBR n (%)	TTP (months)	OS (months)
39	2 (5.1%)	10 (25.6%)	17 (43.6%)	29 (74.4%)	7	14.3

- Treatment regimen: 267mg/m<sup>2</sup> IV infusion every 3 weeks
- Toxicity
  - No cardiotoxicity was observed
  - Hematological toxicity was rapidly reversible
  - Compares favorably to modern triple combination chemotherapy

Ref.: G. Emons et al. EORTC-NCI-AACR 2010



# AEZS-108 Clinical Phase 2

## Endometrial Cancer – Comparison Efficacy

### Randomized Trials of Combination Chemotherapy in Metastatic Endometrial Cancer

Study and Regimen	Number	Response Rate (RR) (%)	Median OS (months)
Thigpen <i>et al.</i> *	356		
Doxorubicin		22	6.7
Doxorubicin/cyclophosphamide		33	7.3
Fleming <i>et al.</i> *	273		
Doxorubicin/cisplatin		34	12.3
Doxorubicin/cisplatin/paclitaxel		57	15.3

**AEZS-108\*\*:** RR = 31%, TTP = 7 months and OS = 14.3 months

\*No patients had received prior therapy with cytotoxic drugs

\*\*Ref.: G. Emons *et al.* ENA 2010

Ref.: Temkin S, Fleming G: Current treatment of metastatic endometrial cancer. *Cancer Control* 2009;16:3

# Clinical Phase 2 – Comparison of ADR Profile with Doxil®

	Endometrial Cancer				Ovarian Cancer				
Drug:	AEZS-108 267 mg/m² q3w (Dox: ≈77)		Doxil 50 mg Dox/m² q4w		AEZS-108 267 mg/m² q3w				
Organ System	Source:	Emons ENA 2010 (n = 43)	Muggia JCO 2002 (n = 43)		Gordon JCO 2001 (n = 239)		Emons ASCO 2010 (n = 43)		
-AE-Term		All Grades	Grade 3/4	All Gr.	Gr. 3/4	All Gr.	Gr. 3/4	All Gr.	Gr. 3/4
Gastrointestinal									
- Nausea		39	2			46	5	79	2
- Vomiting		21	0			33	8	38	0
- Nausea / Vomiting				30	2				
- Diarrhea		9	2			21	3	12	0
- Stomatitis		2	0	(incl. in Mucosal inflammat.)		41	8	0	0
General disorder /administration site									
- Fatigue // Asthenia		25	2			40	7	26	0
- Mucosal inflammation		14	0	33	2	14	4	10	0
Skin and subcutaneous tissue									
- Alopecia		37	0	14	0	19	n.a.	50	0
- Hand-Foot-Syndrome		2	0	40	9	51	24	2	0
- Rash						28	4		
Cardiac disorders									
- Cardiotoxicity		0	0	9	2	0	0	1	0
Nervous system									
- Neuropathy / Neurotoxicity		5	0	14	5	0	0	4	0



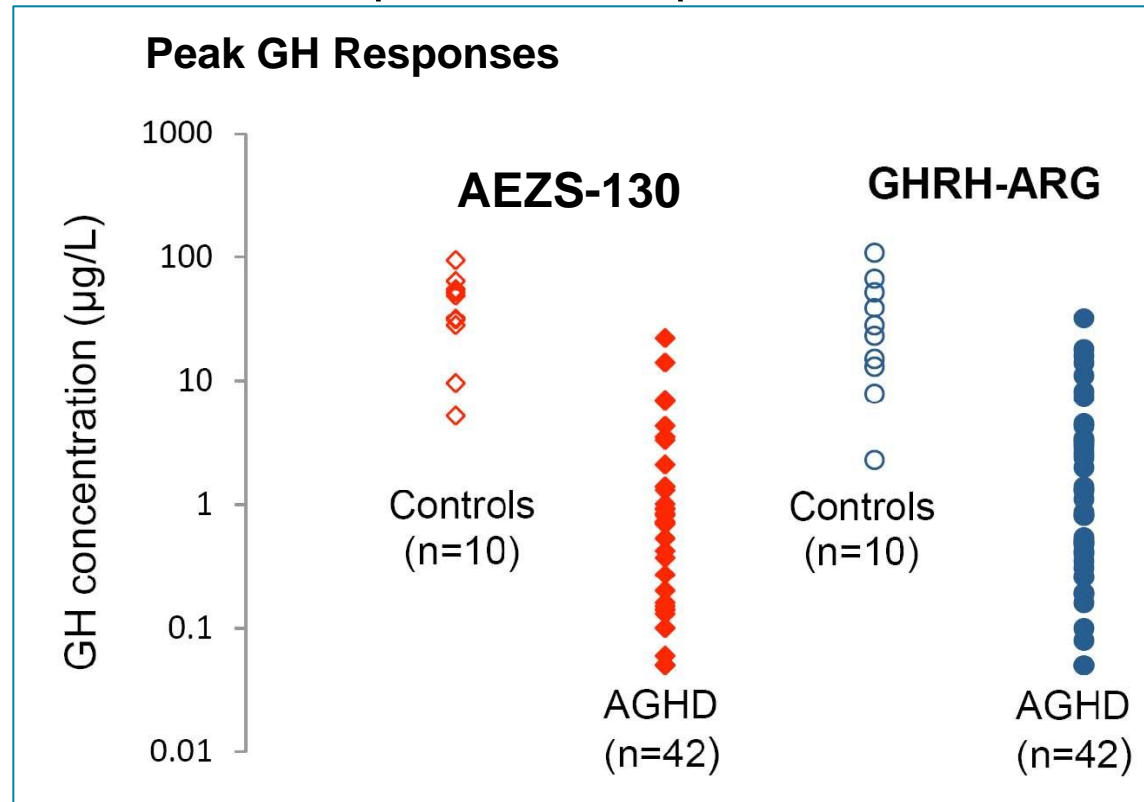
# AEZS-130

# AEZS-130 Overview

- Orally-administered ghrelin agonist
- 100 subjects treated in Phase 1
- Phase 3 as diagnostic test for adult growth hormone deficiency (“GHD”)
  - Orphan drug designation in U.S.
  - SPA granted by FDA in December 2010
- Potential for therapeutic indications
  - Cachexia associated with cancer, COPD and AIDS

# AEZS-130 – Preliminary Phase 3 Results AGHD Diagnostic Test

AEZS-130 resulted in a greater mean separation between the normal controls and AGHD patients compared to GHRH-ARG



Ref.: BMK Biller et al. 2010 5th International Congress  
of the GRS-IGF Society in New York

# AEZS-130 Phase 3 Trial Ongoing Under SPA

- SPA Agreement with FDA
  - Use existing data from the original trial (50 subjects)
  - Include 30 additional control subjects
  - Include 20 additional subjects (10 with AGHD and 10 matched control subjects)
  - All new subjects to receive 0.5 mg/kg body weight of AEZS-130

# Financials

# Consolidated Results

(in millions of US dollars) (unaudited)	For the three months ended	
	March 31, 2011 IFRS	March 31, 2010 IFRS
<i>Revenues</i>		
Sales and royalties	7.1	5.7
License fees and other	0.3	0.7
	7.4	6.4
<i>Operating Expenses</i>		
Cost of sales	6.0	4.6
R&D costs, net	5.5	6.1
SG&A expenses	3.2	3.1
	14.7	13.8
Loss from operations	(7.3)	(7.4)
Net finance (costs) income	(1.9)	1.7
Income tax expense	(0.8)	—
Net loss	(10.0)	(5.7)



# Consolidated Cash Flows (Non-GAAP)

<i>(in millions of US dollars)</i> <i>(unaudited)</i>	<b>For the three months ended March 31, 2011</b>
Cash and cash equivalents – Beginning of period	<b>32.0</b>
Cash flows provided by operating activities	<b>0.8</b>
Cash flows provided by financing activities	<b>5.1</b>
Exchange rate fluctuation impact and other	<b>0.4</b>
Net change in cash and cash equivalents	<b>6.3</b>
Cash and cash equivalents – End of period*	<b>38.3</b>

\* Excluding short-term investment of \$2.8 million

# Selected Information

<i>(in millions of US dollars or of shares) (unaudited)</i>	
Number of shares outstanding as of April 30, 2011	<b>95.2</b>
Price per share as of May 12, 2011	<b>\$2.35</b>
Market capitalization	<b>223.7</b>
Cash, cash equivalent and S-T Investment (no debt) as of March 31, 2011	<b>41.1</b>
Pro forma enterprise value	<b>182.6</b>

Average daily volume shares traded*	<b>Last 3 months</b>
NASDAQ: AEZS	<b>3.2</b>
TSX: AEZ	<b>0.2</b>

\*Source: Yahoo! Finance as of May 12, 2011

# 2011 Milestones

# 2011 Milestones – Perifosine

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- Results for Phase 3 X-PECT trial in refractory advanced colorectal cancer
- Multiple Myeloma Phase 3: additional centers outside U.S.
- Partnering: priority Asia
  - Yakult – Japan ✓
- Decision on the development of new indications such as CLL

# 2011 Milestones – AEZS-108

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- Define regulatory strategy for endometrial cancer with FDA and EMA (Scientific Advice)
- Initiate first pivotal program in endometrial cancer (Phase 3)
- Ongoing Phase 1/2 study in refractory prostate cancer and refractory bladder cancer
  - Update on the studies' progress

# 2011 Milestones – AEZS-130

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- Complete Phase 3 study as diagnostic test for AGHD under SPA in the U.S.
- File NDA as diagnostic test for AGHD in the U.S.
- Start proof-of-concept study in cancer induced cachexia