Southland Times: 1 June, 2017

Southland Times

By Rachel Thomas 1 June 2017

Glowing Tumours Help Patients' Odds

It was by chance that Alice Chambers' brain tumour was found.

The 33-year-old was loading the boot of her jeep when the window slammed down on her head so hard "I thought someone had hit me with a baseball bat". Days later, she noticed a broken blood vessel in her face, and decided to get it checked out, along with a small lump behind her eye.

Following a CT scan, her doctor phoned her with the message: "I'm really sorry, but they've found something".

"I sort of passed the phone to my dad and slid down the wall," she said.

Doctors had found an aggressive, malignant cancerous brain tumour known as a glioblastoma, on the right side of her head.

Two weeks ago, she became the first patient in New Zealand to be treated with a new drug that makes malignant brain tumours glow fluorescent during surgery.

The drug, Gliolan, turns tumours red while the brain mass remains blue, so the tumour can be removed down to macroscopic detail.

Before Gliolan, the average survival rate with treatment for people with standard glioblastomas was 14 months, Wellington Hospital neurosurgeon Kelvin Woon said. "I can say this in front of Alice, because I know it's not going to be that [short] now.

 $^{\prime\prime}\mbox{We got all of the macroscopic tumour out. And she has done very well from it."$

Previously it had been hard for neurosurgeons to distinguish between brain tissue and the edge of the tumour. Not wanting to remove brain tissue, they often ended up leaving parts of the tumour behind.

"The more tumour you remove, the better the outcome," Woon said. "With a lower tumour burden, patients do better and live much longer, and that's what we try to achieve."

Chambers still needs to have chemotherapy and radiotherapy to blast any remnants of the tumour, which has turned out not to be as aggressive as first thought. The surgery has not cured her cancer, but she believes the work of Woon and Gliolan has prolonged her life.

"With the initial prognosis, the idea of never making it to 90 was the worst thing in the world.

"I was looking at my kids and knowing they're 3 and 6 and thinking, if I'm dead in 15 months that's 4 and 7. That's never ever going to be old enough. You can't leave them in the world at that age.

"I can only hope the worst is behind me, and I've got to look forward now and go about my life, and keep going."

A recent Pharmac decision means Gliolan will be available to all district health boards through public funding from June 1. Woon applied to MedSafe to have it made available in 2015, and said the recent Pharmac approval was ''fantastic for New Zealand''.

He estimated about 70 per cent of patients with these particular tumours would be suitable for Gliolan treatment.

Waikato Times: 1 June, 2017

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By Rachel Thomas 1 June 2017

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Brain Surgery Breakthrough: New Zealand Neurosurgeon Pioneers NZ-First Technique

Auckland, New Zealand, 31 May 2017: A 33 year old Wellington mother of two has become the first New Zealand patient to be treated with a novel brain cancer visualisation drug that 'lights up' tumours during surgery to enable more complete removal of the malignant tissue.

GLIOLAN[®] (aminolevulenic acid: ALA) is taken as a drink three hours prior to surgery and works by causing cancerous tissue in the brain to fluoresce. This enables surgeons to more clearly see and better remove highly aggressive brain tumours known as glioblastoma multiforme, or GBM.

The drug will now be reimbursed for New Zealand patients at District Health Boards (DHB) hospitals from tomorrow, **1 June**, following PHARMAC's decision to fund GLIOLAN for newly diagnosed, untreated patients.

It is expected around 100 NZ brain cancer patients a year will now benefit from this cutting-edge medicine, which has been shown to almost double the rate of complete resection and six-month progression-free survival in patients with GBM¹.

The first patient operated on using GLIOLAN is Wellington mother of two Alice Chambers-Smith, who was diagnosed with a brain tumour just weeks ago after moving back to NZ from England with her young family late last year.

Her doctors - who suspected her cancer may be gliolblastoma multiforme - were

able to access GLIOLAN on a compassionate basis prior to the public reimbursement.

The young mother, who has a 3 year-old daughter and 6 year-old son, said she hoped GLIOLAN would enable her doctors to remove as much of her cancer as possible.

"I just want to do every single possible thing I can to be the tiny statistic that doesn't lose this battle," she said.

"I think the PHARMAC decision to make this technology available can only be a good thing."

Leading New Zealand neurosurgeon Mr Kelvin Woon was the first neurosurgeon to use the technology in New Zealand. "GLIOLAN provides a great opportunity for NZ patients who are affected by these highly malignant tumours," he said.

"We are pleased to be pioneering this operation at the Wellington Regional Hospital as we endeavour to improve outcomes for patients with these aggressive brain tumours.

"Although not curative, GLIOLAN helps us to better visualise what can be poorlydefined tumour margins, which limits our ability to resect the tumour macroscopically.

"Using GLIOLAN, we can more clearly see what is brain tissue and what is tumour. This gives us the confidence to be more aggressive and strive for maximum resection. This is important, because the evidence points to maximum (complete macroscopic) resection and increases the chances of extending overall survival." $^{\rm 2}$

GLIOLAN is given to patients as a drink prior to surgery. The drug is preferentially taken up by the malignant tumour tissue.

During surgery, a neurosurgical microscope fitted with a specialised blue operating light is used, which causes cancerous tissue containing the drug to glow fluorescent pink whilst normal brain tissue appears blue. This enables neurosurgeons to better visualise these tumours and more completely remove them, whilst sparing the neighbouring healthy brain tissue.

The drug is made available in New Zealand by international biopharmaceutical company Specialised Therapeutics Ltd, an affiliate of Specialised Therapeutics Asia (ST Asia).

Chief Executive Officer Mr Carlo Montagner applauded the PHARMAC decision to enable GLIOLAN to be used in complex neurosurgery cases for eligible patients.

"In this region and around the world, these patients have typically had a very poor prognosis," he said.

"With current standard chemotherapy and radiation treatment, these patients have a median overall survival of 12, maybe 15 months.³

"GLIOLAN has been shown to help GBM patients survive longer without tumour

progression compared to standard surgical procedures. Any drug or technology that enables patients additional time with their families is extremely valuable."

International studies have shown that the use of GLIOLAN during brain tumour surgery has nearly doubled the rate of achieving a complete resection of the main tumour bulk, which in turn has resulted in a doubling of the number of patients without progression of their brain cancer six months after surgery.¹

The pivotal Phase III study published in The Lancet Oncology Medical Journal reported complete resection of malignant brain tumour tissue in 65% of patients receiving GLIOLAN compared to 36% of patients in the study's control arm (difference between groups 29% [95% CI 17-40], p<0.0001). Six-month progression-free survival was achieved in 41% of patients receiving GLIOLAN compared to 21% of patients who were operated on without the use of the drug (difference between groups 20% [95% CI 9.1-30.7], p=0.0003)¹.

GLIOLAN was first approved in Europe in 2007 and is marketed by medac GmbH in Europe, Africa, South America and Asia (excepting Japan and Korea). Around 500 Australian patients have been operated on using GLIOLAN since 2012.

About GLIOLAN®

The active substance in GLIOLAN, aminolevulinic acid (ALA), is a photoreceptive compound which is absorbed by cells in the body, where it is converted by enzymes into fluorescent chemicals, particularly protoporphyrin IX (PPIX). Since glioma cells take up more of the active substance and convert it more rapidly into PPIX, higher levels of PPIX accumulate in the cancer cells than in normal tissue. When illuminated under blue light of a specific wavelength, the PPIX in the tumour glows an intense red, while the normal brain tissue appears blue. This enables the surgeon to see the tumour more clearly during brain surgery and to remove it more accurately, sparing healthy brain tissue.

Like all medications GLIOLAN may cause side effects. GLIOLAN should not be used in patients with hypersensitivity to ALA or porphyrins, or in cases of acute or chronic porphyria, or in pregnancy. Cardiac disorders, gastrointestinal disorders and skin and subcutaneous disorders are all reported as being uncommon.

About the Specialised Therapeutics Group

The Specialised Therapeutics (ST) group of companies collaborates with leading global pharmaceutical and diagnostic companies to bring novel, innovative and life changing healthcare solutions to patients affected by a range of diseases in Australia, New Zealand and throughout South East Asia. ST is committed to making new and novel therapies available to patients around the world, with a broad therapeutic portfolio spanning oncology, hematology, urology and ophthalmology. Additional information can be found at <u>www.STAbiopharma.com</u>

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- 1. Stummer W, Pichlmeier U, Meinel T, et al., Fluorescence-guided surgery with 5-aminovulinec acid for resection of malignant glioma: a randomised controlled multicentre phase III trial, Lancet Oncol, 2006;7:392-401
- S.J. Hentschel and Sawaya, R. Optimizing Outcomes with Maximal Surgical Resection of Malignant Gliomas. Cancer Control 2003; Vol 10: 109-112
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Our Philanthropic Heart: ST and the Melbourne Coastrek

We are so proud of our intrepid ST foot soldiers, who last week took on the annual Melbourne Coastrek challenge and walked 30 kilometres in the name of charity.

Two teams of ST walkers joined forces to trek along the rugged and spectacular Victorian coastline – a journey that took around 7 hours, large quantities of caffeine and much laughter to complete.

While this was a fantastic team building and bonding experience, an enduring cause is at the heart of this mission.

All funds raised will go to the Fred Hollows Foundation – a development organisation with a simple vision: to put an end to avoidable blindness.

This amazing charity now works in 25 countries around the world and our teams know that sore feet are well worth the effort.

The \$6000 raised by the two teams so far is enough to fund a six month education program for a basic eye doctor in Cambodia, or glasses and frames for thousands of children. Even fundraising just \$4000 can pay for the training of a cataract surgeon in Laos, who will restore sight to thousands of needlessly blind people.

In line with our company's commitment to giving, ST has a Gift Matching Program, where all employee donations made to registered Australian charities are matched dollar for dollar (up to \$1000 per employee per year) by the company.

Under the Sea

Specialised Therapeutics' Spanish partner PharmaMar employs a team of dedicated professional divers who are seeking new solutions for cancer, hundreds of metres below the sea's surface.

We are working closely with our esteemed international partner and have acquired exclusive rights to two cancer compounds of marine origin that are now in late stage development. These compounds may provide new solutions for cancers where there is an unmet need – in multiple myeloma, platinum resistant ovarian cancer and BRCA1&2 mutated metastatic breast cancer, among others.

For a 'deep dive' into PharmaMar's mission, philosophy and expertise, please click on the following video link.



Specialised Therapeutics Asia to License a Promising Anti-Cancer

CompoundLurbinectedin(PM1183) for South East Asia,Australia and New Zealand

SINGAPORE and MELBOURNE, Australia, May 17, 2017: International biopharmaceutical company Specialised Therapeutics Asia (ST Asia) is set to commercialise a promising new anti-cancer drug throughout South East Asia, after signing a second major licensing deal with European pharmaceutical company PharmaMar.

The latest agreement allows ST Asia marketing and distribution rights to new anti-cancer compound lurbinectedin (PM1183) in Australia, New Zealand and throughout SE Asia.

This promising agent is currently in final stage (Phase 3) trials as a potential new therapy for various solid tumours, including platinum-resistant ovarian cancer and small cell lung cancer. In addition, it is in a Phase 2 trial for metastatic breast cancer with BRCA1 and BRCA2 mutations.

Commercial terms of the new license agreement are not being disclosed by ST Asia, but PharmaMar will receive an upfront payment, royalties and additional remunerations for regulatory and sales milestones achieved in these new markets.

An ST Asia affiliate company will also make an equity investment in PharmaMar.

PharmaMar will also retain development and production rights for lurbinectedin (PM1183), and pending completion of all regulatory processes, will supply the finished product to ST Asia for exclusive commercial use in all agreed regions.

ST Asia Chief Executive Officer Mr Carlo Montagner said this new licensing deal cemented the company's existing strong relationship with PharmaMar and demonstrated high confidence in the partner company's development pipeline.

"We have the highest regard for PharmaMar and are pleased to partner once again, pursuing development of this highly promising oncology compound," he said. "We eagerly await data from these final stage studies and look forward to making new therapies like this available to patients throughout our regions who are affected by difficult to treat cancers."

Lurbinectedin (PM1183) is the third marine-derived organism in development by PharmaMar.

Data from the Phase 3 study of lurbinectedin (PM1183) in resistant ovarian cancer (CORAIL) is expected to be available later this year, following the completion of patient recruitment in October 2016.

A Phase 3 trial in small cell lung cancer (ATLANTIS) was initiated in August 2016.

PharmaMar Chairman José María Fernandez Sousa-Faro said: "We are proud to enter into a new agreement with ST Asia, enabling us to reach new populations of cancer patients who may benefit from our novel therapies.

"We remain committed to advancing the development of innovative therapies that may benefit society."

About Specialised Therapeutics Asia

Specialised Therapeutics Asia Pte Ltd (ST Asia) is an international biopharmaceutical company established to provide pioneering healthcare solutions to patients throughout South East Asia, as well as in Australia and New Zealand. The company is a close affiliate of Specialised Therapeutics Australia (STA), which also collaborates with leading global pharmaceutical and diagnostic companies to bring novel, innovative and life changing healthcare solutions to patients affected by a range of diseases. ST Asia is committed to making new and novel therapies available to patients around the world, with a broad therapeutic portfolio spanning oncology, haematology, urology and ophthalmology. Additional information can be found at www.STAbiopharma.com

About PharmaMar

Headquartered in Madrid, PharmaMar is a world-leading biopharmaceutical

company in the discovery and development of innovative marine-derived anticancer drugs. The company has an important pipeline of drug candidates and a robust R&D oncology program. PharmaMar develops and commercialises YONDELIS® in Europe and has three other clinical stage programs under development for several types of solid and haematological cancers PM1183, plitidepsin, and PM60184. PharmaMar is a global biopharmaceutical company with subsidiaries in Germany, Italy, France, Switzerland United Kingdom, Belgium and the United States. PharmaMar fully owns three other companies: GENOMICA, Spain's leading molecular diagnostics company; Sylentis, dedicated to researching therapeutic applications of gene silencing (RNAi); and two other chemical enterprises, Zelnova and Xylazel. To learn more about PharmaMar, please visit us at www.pharmamar.com.

About lurbinectedin (PM1183)

PM1183 is a compound under clinical investigation. It is an inhibitor of RNA polymerase II. This enzyme is essential for the transcription process that is overactivated in tumours with transcription addiction. The antitumour efficacy of lurbinected in is being investigated in various types of solid tumours.

Disclaimer

This document is a press release, not a prospectus. This document does not constitute or form part of an offering or invitation to sell or a solicitation to purchase, offer or subscribe shares of the company. Moreover, no reliance should be placed upon this document for any investment decision or contract and it does not constitute a recommendation of any type with regard to the shares of the company.

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STA Communications Manager Emma Power - +61 419 149 525

Brain Tumour Visualisation Drug GLIOLAN to be Listed on NZ Hospital Medicines List from 1 June

Singapore, Melbourne and Auckland, 28 April 2017: A NOVEL drug which 'lights up' malignant brain tumours to help surgeons more thoroughly resect the cancer tissue will be widely available to New Zealand patients from **1 June**, after a leading neurosurgeon applied for its reimbursement.

The drug, GLIOLAN (aminolevulinic acid HCl), assists neurosurgeons to more completely remove malignant brain tumours (gliomas) by causing them to become fluorescent during surgery.

It is expected around 100 NZ brain cancer patients a year will be operated on using this cutting-edge technology, which has been demonstrated to improve complete resection rates and almost double six-month progression free survival in patients with the most serious form of brain tumours, Glioblastoma Multiforme, or GBM¹.

It will be made available to newly diagnosed, untreated patients who are eligible for fluorescence-guided surgery.

GLIOLAN will be reimbursed subject to the following hospital restrictions:

- Patient has newly diagnosed, untreated, glioblastoma multiforme
- Treatment to be used as adjuvant to fluorescence-guided resection
- Patient's tumour is amenable to complete resection

Leading New Zealand neurosurgeon Dr Kelvin Woon made an application to PHARMAC seeking reimbursement and ensuring GLIOLAN's broad accessibility.

He has described the PHARMAC decision to list GLIOLAN on the hospital

medicines list as "a big step forward".

"This is a great opportunity for NZ patients who are affected by these highly malignant tumours," he said.

"Although not curative, GLIOLAN helps us to better visualise what can be poorlydefined tumour margins, which limits our ability to resect the tumour macroscopically.

"Because we can more clearly see what is brain tissue and what is tumour, it gives us the confidence to be more aggressive and strive for maximum resection. This is important, because the evidence points to maximum (complete macroscopic) resection and increases the chances of overall survival." 2

GLIOLAN is given to patients as a drink prior to surgery. The drug is preferentially taken up by the malignant tumour tissue.

During surgery, a neurosurgical microscope fitted with a specialised blue operating light is used, which causes cancerous tissue containing the drug to glow fluorescent pink whilst normal brain tissue appears blue. This enables neurosurgeons to better visualise these tumours and more completely remove them, whilst sparing the neighbouring healthy brain tissue.

The drug is made available in New Zealand by international biopharmaceutical company Specialised Therapeutics Ltd, an affiliate of Specialised Therapeutics Asia (ST Asia).

Chief Executive Officer Mr Carlo Montagner said several NZ hospitals had already upgraded operating theatre equipment to enable the use of GLIOLAN and neurosurgeons were preparing to use this technology as soon as the PHARMAC approval and listing takes effect.

"We are delighted to be able to provide another tool for NZ neurosurgeons to use in complex brain tumour cases," he said.

"In this region and around the world, these patients have a very poor prognosis. With current standard chemotherapy and radiation treatment, these patients have

a median overall survival of 12, maybe 15 months.³ GLIOLAN has been shown to help GBM patients survive longer without tumour progression compared to

standard surgical procedures. Any drug or technology that enables patients additional time with their families is extremely valuable."

International studies have shown that the use of GLIOLAN during brain tumour surgery has nearly doubled the rate of achieving a complete resection of the main tumour bulk, which in turn has resulted in a doubling of the number of patients

without progression of their brain cancer six months after surgery. $^{1} \ \ \,$

The pivotal Phase III study published in The Lancet Oncology Medical Journal reported complete resection of malignant brain tumour tissue in 65% of patients receiving GLIOLAN compared to 36% of patients in the study's control arm (difference between groups 29% [95% CI 17-40], p<0.0001). Six-month progression-free survival was achieved in 41% of patients receiving GLIOLAN compared to 21% of patients who were operated on without the use of the drug

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GLIOLAN will be available to purchase from May 12 from ST's New Zealand distributor, Healthcare Logistics (HCL).

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For all inquiries, please phone Specialised Therapeutics Asia Communications Manager Emma Power on +61 149 149 525

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ST Supporting Private Cancer Physicians of Australia



DR HILDA HIGH - PRIVATE CANCER GENETICIST.

While Dr Hilda High was always intent on a career in medicine, her professional life took a few tangents before her true calling again beckoned. Following a dietetics degree and experience in computer programming, Hilda began her medical training relatively late in life, at age 32. Now, at 50, she is among Australia's telehealth pioneers, after establishing a private cancer genetics clinic in NSW four years ago. Last year she consulted with more than 200 families around the country, sometimes face to face and sometimes remotely. She describes cancer genetics as endlessly fascinating and concedes that working in a private practice has enabled unrivalled flexibility, both personally and professionally. Still, finding her niche has not been without its challenges.

Tell us about your career path and how you came to choose cancer genetics?

Medical oncology was something I had always wanted to do, from the day I started as a medical student. Cancer genetics was something I only became aware

of in my final year of specialist medical oncology training. And I absolutely love it. There are a couple of reasons I went into cancer genetics instead of medical oncology. I was a sole parent – a sole parent with a young daughter. I did not want on-call, overtime or weekends. And cancer genetics has no medical emergencies. The second reason is, I find it intellectually fascinating as well as being a preventative form of medicine. That it turned out to be suited to Telehealth was a lucky bonus. Basically, it ticks all the boxes for me. The reason I went into private practice was in part, because cancer genetics allowed me to have no on-call obligations. Also, I found a niche doing telehealth to rural families, something that was relatively easy in private practice but can be complicated to do from a public hospital setting.

What would you have liked to know before moving into private practice?

Nobody tells you about the ancillary costs of running your own business. Things like the IT costs, setting up telephones, organising premises, those sorts of things and the hiring and firing staff: all of the parts that nobody teaches you at medical school.

Many doctors today still don't understand how their billings work, how Medicare works – because they don't have to. It gets done for them behind the scenes.

Because I work privately, and because I do Telehealth, I need to understand my own billings and that means understanding Medicare very well. But it's not something that was ever formally taught. Understanding how telehealth worked, how billings had to be set up, was essential to establishing a successful private practice.

Should advanced trainees have some business training before working in private practice?

I think it should be part of every placement. Advanced trainees should be asking their bosses – working in either public or private practice — what the costs are. Because even if you are a head of department in the public setting, you will still have to deal with a budget, manage staff and deal with software. I think advanced trainees overall tend to concentrate on learning their specialty. I would say to them, 'you should spend an equal amount of time learning to run a practice, public or private'.

So what advice you would give advanced trainees?

One thing that is very helpful is to do a fellowship year or to start by working as a locum, or by somehow attaching yourself to an experienced established oncologist.

I did a fellowship year. This meant spending a year working as a specialist, but still under the umbrella, or the care of a consultant who was running a public clinic. And that was much more useful than when I was a registrar. Towards the end of the year I realised there was no suitable position for me anywhere in Australia given that cancer genetics is a small field. So, I had to make a decision either to set up my own practice or to find a position as a medical oncologist. That's when I registered Sydney Cancer Genetics as a business name and committed to setting up my own cancer genetics private practice.

I took a room in an existing and very supportive practice, working for a percentage of earnings. When I started my telehealth practice, I ran it from a home office. That kept costs low and meant that I wasn't going to lose money (if you are paying room rental you can earn less than nothing!).

What else helped you along the way and what advice would you impart to current advanced trainees?

Mentors. I strongly recommend all advanced trainees find at least one mentor. They may not be in the same speciality, although that helps and may not be in the same city as you. They are someone who you can bounce ideas off and be supported by.

Another tip: If possible, it's great when you are starting out to work for a percentage of your earnings and use the infrastructure of an existing practice – things like their patient database, their transcription service, their billing service. This allows you to find your feet as a consultant and then start to learn the 'ins

and outs' of running a business.

When you are spending time with different doctors, you are being exposed to different management styles, different practice styles, and different patient software. Even learning how not to do things is useful!

Also, work for someone else first. Most practices need locums, so while you are doing that, you can be passively or actively acquiring that information. I have found people are incredibly generous with their time and their support. If you are having problems finding that right person, find a mentor who is not in competition with you. For example, someone who lives and practices in a different state. I am glad I took the time to really learn the behind the scenes things and to really understand every aspect of running my practice.

Best thing about private practice?

I think going into private practice is a personality match as much as anything. I could work within the public system, but private practice gives me significantly more personal flexibility such as around holidays and working hours as well as the ability to move the business in new directions to address patient needs and opportunities much more rapidly than I would be able to in the public system.

In my clinic, I am a sole practitioner. The beauty of private practice – if you become too busy, you can employ somebody else to work with you.

What does the future hold for advanced trainees, in your opinion?

It is going to be important to find a niche. I think there is going to be an oversupply of specialists wanting to work in large centres. I think there is going to be a greater push to make public hospital specialists work for billings, which means removing the flexibility of private practice and removing the security and perks (such as annual leave/ super / conference leave etc.) of a public position at the same time. Specific to Medical Oncology, I think the number of treatments and the way we approach cancer is going to continue to change with a much greater emphasis on the molecular genetics of the cancers.

Final words?

I am extremely glad I moved into private practice. I don't think it is as lucrative as a secure quality position in the public system (if such a thing exists!). But it has given me an amazing amount of freedom and a huge amount of personal and intellectual satisfaction.

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.-(<u>BUSINESS WIRE</u>)-Aeterna Zentaris Inc. (NASDAQ: AEZS) (TSX: AEZ) (the "Company") today announced the occurrence of the 384th death in the pivotal Phase 3 ZoptEC (**Zop**tarelin Doxorubicin in Endometrial Cancer) 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 <u>www.aezsinc.com</u>.

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 forwardlooking statements. The Company does not undertake to update these forwardlooking 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.

Julie's Ordeal with Oropharyngeal Cancer and Oral Mucositis

"I was diagnosed in 2013. Most throat cancers are caused by alcohol and tobacco, but mine was caused by the human papillomavirus (HPV). The primary site was on my tonsils, on the back of my tongue and on the side of my throat. My treatment was 30 consecutive days of radiation therapy. This meant a 20 minute radiation session every day, plus weekly chemotherapy. Basically, when you have radiation, you cook from the inside out. The impact on the inside of my mouth was catastrophic.

I lost over 20 kilograms in six weeks. That is a common issue with oral cancers. I didn't get tube fed. I kept taking nutrition orally, but was on liquid food for a number of weeks. I was able to keep swallowing enough liquid food to stay alive,

basically. The inside of my mouth became very burnt. Under your tongue there are little lines of flesh and they swelled up like great big fingers of swollen soft flesh. My mouth was traumatised. It was very sore inside, I had ulcers, flesh sloughed off. It took a good six months before my mouth was anything close to normal.

These days, I am much, much better, I am back working normally and I eat normally. I lost the capacity to sing, and I used to have quite a nice voice. It's not my old voice, but I can speak. I am an example of radiation and chemotherapy being absolutely worth the effort."