Company Update – Dec 2016

See below, Specialised Therapeutics' Company Update for December 2016. To view this publication, move your mouse over the image below and click on the Fullscreen button in the top right corner to expand the window.

The PBS 'Price' Should Never Influence Cancer Treatment

August, 2016: In Australia, the cost of treating some cancers is undoubtedly becoming more expensive.

Physicians frequently express concern about the cost of prescribing innovative branded medicines because of the significant taxpayer contribution required to fund the PBS.

Further, they concede that they *are* mindful of the public purse when it comes to this sort of decision making.

Just last week at the Australian Lung Cancer Conference in Melbourne, the term "financial toxicity" was used in the plenary session to describe one of the issues oncologists will be faced with when prescribing novel immuno-oncology agents.

Putting the complex issue of health economics aside, drug prescribers should be aware that the price they are presented with when reviewing the PBS schedule is unlikely to be the 'real' price, and that the 'true' price to the taxpayer is most likely far less.

When headlines scream that a new drug costs $150,000 per year to treat a particular disease, the reality is that the actual cost to the taxpayer will be substantially less – up to 50% less – because it is highly likely that the PBS authorities have negotiated a confidential *Special Pricing Arrangement* with the

pharmaceutical company well in advance.

In addition, many recent PBS listings of novel branded medicines include *Risk Share Arrangements* where price rebates and/or the use beyond predetermined prescription thresholds trigger substantial rebates back to the Commonwealth.

Leading pharma industry publication Pharma Dispatch recently reported that the size of PBS rebates and discounts from PBS listed drugs which have a *Risk Share Arrangement* in place has risen from $50M in 2009-10 to over $700M in 2014-15. Further, this is projected to top $1 billion this financial year, on a projected total PBS outlay of approximately $10 Billion.

These arrangements are highly confidential as there are many local and international pricing implications. Without such confidentiality I doubt many of these novel agents would ever be listed in low drug priced countries such as Australia.

STA's oncology drug Abraxane is one such anti-cancer agent that is subject to both *Special Pricing* and *Risk Share Arrangements.* The price of Abraxane as listed on the PBS website is not the price the taxpayer is paying. Not even close — particularly if Abraxane's prescription level exceeds pre-specified thresholds.

From a company and taxpayer perspective, this means that each quarter STA, like many other pharma companies in Australia, reimburses the PBS to the tune of hundreds of thousands of dollars.

On average it takes a pharma company 2-3 PBAC submissions over several years to achieve a PBS listing for an anti-cancer agent. This involves highly complex health economic analyses and ultimately, pricing/rebate negotiations.

So when any drug finally makes it on to the PBS, physicians should have the confidence that the PBS has extracted maximum value from the pharma company, even though on the PBS listing website, the price appears to be expensive or even excessive. There is simply no need for any physician to potentially conduct a secondary 'cost to the community' analysis of a novel expensive agent when deciding which agent to use, as many of the 'true' cost inputs are not available to the public – such an analysis only serves to undermine the complexity and integrity of the initial PBS listing process.

So when it all boils down to it, Australians who have worked hard and spent a lifetime of paying taxes should be entitled to access any drug that is PBS listed for their specific condition – even if there is a seemingly less expensive alternative. Equitable access to all PBS listed cancer drugs is a hard earned basic right for all Australians and is priceless.

Herald Sun: 5 November, 2016

HERALD SUN (MELBOURNE)

By Grant McArthur 5 November 2016

Linda Defies Odds:
Brave cancer survivor is determined to live life to the full

FEW people would laugh about their superannuation running out while they still have a lot of living left to do.

But for Linda Wilson, the prospect of outliving her nest egg by defying pancreatic cancer is a dream worth celebrating.

Diagnosed with terminal cancer in early 2012, the Somers 59-year-old's prognosis was so bleak she was granted immediate access to her superannuation, though not some of the drugs hoped to prolong or improve her life.

But through surgery, two rounds of chemotherapy and finally an expensive

yearlong course of new drug Abraxane, she has so far defied the disease that claims 93 per cent of its sufferers
within five years.

Ms Wilson has travelled Australia to go fishing, ticked items off her bucket list and bought a new car while continuing to work as a nurse.

Now she is also hoping to fight for greater attention and research for more than 3100 Australians diagnosed with pancreatic cancer each year.

“In 2013 I was told I had approximately 6-12 months to live when I asked the question, but I didn’t really like the answer,” Ms Wilson said.

“I am unlucky to get pancreatic cancer, but I tell you, the last 4½ years I have never lived so much.”

In 2014 Abraxane was placed on the Pharmaceutical Benefits Scheme of pancreatic cancer as a frontline therapy, but because she had already undergone other chemotherapies the $1300-afortnight treatment was not subsidised for her.

Other bone marrowboosting injections were also not provided for Ms Wilson because she was deemed terminal.

Fortunately for Ms Wilson, the charitable John Logan Foundation stepped in to fund her Abraxane treatment through 2015.

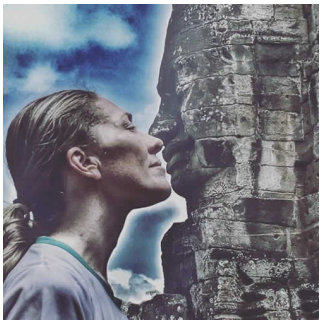
As Pancreatic Cancer Awareness Month kicks off this week, Ms Wilson is also preparing for a new battle. Recent scans have revealed a 2cm mass on her pancreas indicating the cancer
has returned, and doctors at The Alfred are now trying to determine if surgery to remove the organ could be successful in getting the entire cancer, or whether further chemotherapy is the best cause of action.

“That is where we are at the moment. But don’t worry, I don’t give up easily,” Ms Wilson said.

Sports Broadcaster Takes on New Sporting Challenge

Specialised Therapeutics Australia (STA) has a particular interest in pancreatic cancer and is committed to supporting efforts to advance understanding and improve clinical outcomes in this disease.

Testimony to this commitment, we recently sponsored well-known television personality and sports broadcaster Tiffany Cherry to participate in the GI Cancer Institute's 'Gutsy Challenge' – a seven day cycling challenge across Cambodia to raise awareness and vital funding for Australian research into GI cancers.



Monies raised by our rider Tiffany are being specifically directed into a fund for Australian pancreatic cancer research.

Tiffany rode as part of a cycling team including oncologists, cancer survivors and others who have been touched by GI cancers.

STA shared highlights of Tiffany's Cambodian journey on our LinkedIn page. She interviewed others in her group and shared their stories. We encourage you to follow STA and like and share our posts by clicking here.

STA was proud to support this fantastic initiative. For more information about the GI Cancer Institute click here.

First Australian Patients Treated with New Brain Tumour Drug – Gliolan®

Melbourne, Australia: Two Australian patients have undergone surgery with the aid of a new drug which assists neurosurgeons to visualise and remove high grade gliomas – brain tumours which typically have a poor prognosis.

Neurosurgeons at Brisbane's Wesley Hospital and the Royal Melbourne Hospital have pioneered the use of Gliolan® (5-aminolevulinic acid), describing it as "breakthrough technology".

The drug is given to patients three hours prior to surgery and causes cancerous tissue to glow fluorescent red during brain surgery. This enables improved visualisation of the boundary between healthy and diseased brain tissue, and aids the surgeon to more thoroughly remove the tumour. International studies have shown the use of Gliolan during surgery has nearly doubled the rate of achieving

a complete resection, and has doubled the number of patients without progression of their brain cancer six months after their surgery.[1]

Melbourne bio-pharmaceutical company Specialised Therapeutics Australia Ltd (STA) has licensed the drug for use in Australia and New Zealand. CEO of STA, Carlo Montagner said the availability of this drug in Australia will potentially benefit hundreds of brain tumour patients.

“We are pleased to make Gliolan available to Australians and expect it to be readily adopted by the neurosurgery community, so patients around Australia may benefit from the most sophisticated available technology,” he said.

Mr Montagner added: “We have made clear our strategy of building Specialised Therapeutics Australia through the recent acquisition and continued growth of several specialist medicines which offer unique clinical benefits to patients.”

The pivotal Phase III registration study published in The Lancet Oncology medical journal demonstrated complete resection of malignant high grade glioma tissue in 65% of patients receiving Gliolan, compared with 36% of patients in the control arm. Additionally, 6-month progression-free survival was achieved in 41% of patients receiving Gliolan compared to 21.1% of patients who were operated on without the use of the drug.[1]

The principal investigator of this pivotal trial, German neurosurgeon Professor Walter Stummer, has been in Australia to provide education and training using Gliolan for fluorescence-guided surgery. Nineteen Australian neurosurgeons are now trained and have been certified in this method, which will enable them to offer fluorescence-based resection of brain tumours to their patients.

Melbourne neurosurgeon, Dr Kate Drummond from the Royal Melbourne Hospital commented: “We hope that by using this breakthrough technology we will be able to improve the outcomes for Australian brain tumour patients.”

“In my first surgery using this drug, we found additional pockets of fluorescent pink tissue that I was able to remove, and that I may not have seen in a routine surgery. Using Gliolan allowed me to see the contrast of the pink and red fluorescing tumour tissue compared to the healthy non-fluorescing brain tissue. I was able to protect the normal brain tissue from damage during the surgery. ”

“We are pleased to be pioneering this operation at the Royal Melbourne Hospital and expect our neurosurgery colleagues around the country to follow suit.”

The active substance in Gliolan, 5-aminolevulinic acid, 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).[2]

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.[2]

The drug is approved for use in 27 countries across Europe, and Korea. Gliolan was first approved in Europe in 2007 and is marketed by Medac in Europe, Africa, South America and Asia (excluding Japan and Korea). Gliolan is not yet approved by the Therapeutic Goods Administration (TGA). The drug will be made available to Australian neurosurgeons who have undergone training, through the federal government’s Special Access Scheme (SAS) until TGA approved.

References:

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
2. European Public Assessment Report

About Gliolan®

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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.[2]

Like all medications Gliolan may cause side effects. Gliolan should not be used in patients with hypersensitivity to 5-ALA or porphyrins, 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 Specialised Therapeutics Australia, Pty Ltd

Specialised Therapeutics Australia Pty Ltd (STA) was established to identify, develop and commercialise innovative anti-cancer and other specialised therapies for the Asia-Pacific market. Currently STA markets two world leading cancer and cancer supportive care therapies, ABRAXANE® (nab-paclitaxel) and ALOXI® (palonosetron) respectively. Based in Melbourne, Australia, the privately held company is currently developing several more important therapeutic agents for release in the Asia-Pacific region.

Aeterna Zentaris and Specialised Therapeutics Asia Sign Exclusive License Agreement for the Potential Marketing of Zoptrex™

in Australia and New Zealand

Charleston, South Carolina and Singapore, October 12, 2016: Aeterna Zentaris Inc. (NASDAQ: AEZS, TSX: AEZ) (the "Company") and Specialised Therapeutics Asia ("STA") today announced the signing of an exclusive license agreement for the Company's lead investigational anti-cancer compound, Zoptrex™ (zoptarelin doxorubicin), for the territories of Australia and New Zealand (the "Territory"). Zoptrex™, a novel synthetic peptide carrier linked to doxorubicin, is currently undergoing a fully-enrolled Phase 3 clinical trial to evaluate the compound in endometrial cancer. The Company expects to complete the Phase 3 clinical trial in 2016 and, if the results of the trial warrant doing so, to submit a new drug application for Zoptrex™ to the United States Food and Drug Administration (FDA) in the first half of 2017. Zoptrex™ is the Company's proposed tradename for zoptarelin doxorubicin. The proposed tradename is subject to approval by the FDA.

Under the terms of the License Agreement, Aeterna Zentaris will be entitled to receive a non refundable upfront payment in consideration for the license to STA of the Company's intellectual property related to Zoptrex™ and the grant to STA of the right to commercialize Zoptrex™ in the Territory. STA has also agreed to make additional payments to the Company upon achieving certain pre-established regulatory and commercial milestones, as well as double-digit royalties on future net sales of Zoptrex™ in the Territory. STA will be responsible for the development, registration, reimbursement and commercialization of the product in the Territory. The Company and STA have also entered into a supply agreement, pursuant to which the Company will supply Zoptrex™ to STA for the duration of the license agreement.

David Dodd, President and CEO of the Company, stated, "I am very pleased that we have now concluded four agreements for the commercial rights to Zoptrex™, if approved, outside the United States. We believe that the interest in Zoptrex expressed by our licensees supports our view that Zoptrex™, if it is approved by the FDA for its initial indication, could be an important treatment option for women with the most severe form of endometrial cancer. We are particularly pleased to have a company of the caliber of STA as a licensee. STA enjoys the

highest reputation in its markets and, with its existing portfolio of oncology products, it has the capability to position Zoptrex™ very well in the market."

STA Chief Executive Officer Mr. Carlo Montagner said Zoptrex™ had demonstrated great potential and was poised to add further value to the company's expanding oncology portfolio. "All results to date suggest Zoptrex™ is a potent new compound and we look forward to collaborating closely with Aeterna Zentaris to maximise its full potential in our key markets," he said.

About Zoptrex™

Zoptrex™ (zoptarelin doxorubicin) is a complex molecule that combines a synthetic peptide carrier with doxorubicin, a well-known chemotherapy agent. The synthetic peptide carrier is (D)-Lys6-LHRH, a modified natural hormone believed to have a strong affinity for 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. Potential benefits of this targeted approach include enhanced efficacy and a more favorable safety profile with lower incidence and severity of side effects as compared to doxorubicin.

About Specialised Therapeutics Asia

Specialised Therapeutics Asia Pte Ltd ("STA") 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, 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. STA 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 www.specialisedtherapeutics.com.au.

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 are now conducting Phase 3 studies of two internally developed compounds. The focus of our business development efforts is the acquisition or license of 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 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.

PBS Price Cuts Undermine Long Term Innovation

Earlier this week, pharmaceutical companies across the country copped a hefty financial blow, as the Federal Government's PBS cost saving agenda took flight.

Under this plan, expected to save the Federal Government around $3.7 billion over the next five years, all branded medicines that have been listed on the PBS for five years or more will be available to the Government at 5% less than it has paid to date.

While taxpayers will see a difference in the price of high volume generics (known as F2 medicines) under this plan, those prescribed specialty branded medicines will not see any difference at the pharmacy counter. That's because a patient's co-payment will remain the same regardless of what the Government pays the pharma company.

This decision will affect dozens of branded medicines and almost every pharmaceutical company in Australia.

The general perception is that pharma companies can afford it. But behind the scenes, and what many Australians don't understand, is that pharma companies are innovators, educators and philanthropists, consistently funding important clinical trials of new drugs that may change lives, educating the medical community about new technologies and providing millions of dollars in financial support to health programs and initiatives.

In addition, pharma companies consistently support extensive compassionate programs enabling patient access to specialty medicines not yet listed, that might otherwise be unaffordable.

Not every drug makes it to market. For every innovative therapy that becomes available and changes lives, there are hundreds of others that fail – in some cases, after millions of dollars have been spent in development. This is not a waste. Many a brilliant discovery was made on the back of a litany of supposed 'failures'.

This is the reality of innovation and yes, it costs money.

While positive for the Commonwealth drug budget, these latest PBS cost saving measures pose an unprecedented commercial challenge for the pharmaceutical industry and in particular, for innovator pharma companies.

Remember, Australia's pharma industry invests over $1 billion every year in health and medical research, exports billions in manufactured goods and indirectly employs around 20,000 Australians.

It is vital this industry is financially supported. This is the first forced price cut introduced by the Government – we don't know if it will be the last.

If prices are further reduced, there is a real possibility pharma companies will remove some specialty branded medicines from the PBS because it won't be commercially viable – companies may not be able to sustain the supply of their products to the Government at a reduced rate in the long term.

If this happens, cutting edge, life saving drug therapies currently listed and being used to treat Australian patients may become unavailable – and this is a great pity

for our community.

There is little doubt PBS pricing changes **WILL** impact innovation. Trials of new drugs are costly and pharma companies will simply not have the same commercial incentive to include Australian sites in global studies of new drugs and technologies.

This is disappointing. Not only does it deny patients the opportunity to receive innovative drugs, potentially life changing therapies, it also fails to recognise the massive economic boost these trials provide to Australia in terms of funding and employment at trial hospitals and other academic and research institutions. These programs create employment for scientists and researchers and contribute to our 'knowledge economy'.

Further, the market prices able to be achieved with new therapies currently in development will be benchmarked against reduced PBS prices from 1 April.

Effectively, it will be more difficult for new innovator drugs to achieve Government reimbursement because the innovation-driven development companies will not be able to match the eroded price or their new therapy will need to achieve almost impossible clinical improvements to justify the same price achievable elsewhere in the world.

The community should also be aware that for those at the commercial coal face – the pharma companies – the opportunity to increase prices once a drug has been listed on the PBS is non-existent.

We wear the costs of any manufacturing price rises or any significant currency devaluations from when a drug is listed, unlike private health insurers for example who are accustomed to achieving in excess of CPI price increases each year.

Pharmaceutical companies like ours need incentives to invest in new therapies, contribute to local and international clinical trials and also, to pay the substantial upfront licensing, acquisition and regulatory fees required to provide cutting edge therapies to the community.

The reality is that to remain competitive and keep innovating, the pharmaceutical industry must be incentivised to continue investing in Australia.

I ask the Government to remember its commitment to innovation. A truly innovative economy and pharmaceutical industry requires the financial ballast to achieve.

Shelli’s Story: How Breast Cancer Inspired ‘Kit for Cancer’

Two and a half years ago, Melbourne based high flyer Shelli Whitehurst was diagnosed with Stage 4 breast cancer. The devastating diagnosis inspired her to found a business that's now helping other cancer patients around the world.

Shelli Whitehurst was at the pinnacle of her career, enjoying the success of the marketing business she founded when her life changed in an instant.

It was May 2014. Her strategic digital marketing company 'Code Name Max' was generating millions of dollars, she was working 15 hours a day, doing business in New York and loving every second.

She had experienced some pain – it was dismissed for months as an infection – then she visited an ophthalmologist with an eye so sore it was difficult to see.

Further investigation revealed two tumours behind each of her eyes and they were not primary cancers.

“My diagnosis was very rapid and very rare,” Shelli reflects.

“I was in New York, doing business, at the top of my game. I was a business owner, an entrepreneur, doing the whole thing and literally within seconds, my world just stopped.

“I could not get into a doctor for six weeks and I was having a mental breakdown, because when you put into ‘Doctor Google’ that cancer has left the primary area, you know that is not a good sign. You don’t die of Stage 1 to 3 cancer anymore, you die of Stage 4.”

Through a friend of a friend, Shelli was able to book into a breast surgeon a little earlier than expected. A day of extensive medical tests followed and she soon received terrible news: the cancer was in both breasts and her bones. She had rib and shoulder fractures. Her breast tumours were massive.

“I was full of cancer – when they scanned me I lit up like a Christmas tree,” Shelli says.

“You go from being a person to a patient. I had never been sick in my life.”

Because the cancer had spread so far, it was decided a double mastectomy would be of very little benefit.

“So we decided not to do surgery but we would monitor everything very closely and do surgery when required.”

She was put on to a new drug that was not yet listed on the Pharmaceutical Benefits Scheme and subsidised by the Federal Government. It meant she had to come up with tens of thousands of dollars within days.

Shelli has since had seven lines of therapy, including immunotherapy.

Unfortunately, despite the hope of immunotherapy, it did not work for Shelli. Now, her cancer has spread to her brain, lungs and liver.

“There is no drug that will fix me because my cancer is so weird and rare. I am 18 months past my expiry date. Now, we are on a different system of drugs which attack the cancer in a different way – which is good – and we

have had five months of stability."

These days, Shelli remains a vital part of the business she founded, but concedes she works nowhere near the number of hours she once did.

She has moved home with her Mum and Dad and relishes spending time with her four year old niece Lyla and one year old nephew, Hudson. Travelling with them to Disneyland is really the only thing on her bucket list.

"I am 41 now. I have an incredible family and I love being around them. I try to be positive. It takes just as much energy being sad as being happy."

At the moment, her entrepreneurial energies are diverted not only into her original company Code Name Max, but also into a new business she founded just days after her diagnosis.

Kit for Cancer is an enterprise committed to helping other cancer patients by developing and selling custom-made care packages to support them while they undergo treatment.

Kit for Cancer also collaborates with corporate partners to custom design kits for specific patient groups.

It's an online business operating from her mother's home and Shelli has sold hundreds of kits around the world, including in Zimbabwe and Brazil.

Testimony to her drive, the business is rapidly growing and evolving into The KIT Foundation.

Shelli says "Kit" began when she realised that there was no one-stop online shop "to have everything I needed" delivered when she became sick.

"People send you flowers and it's lovely. While none of those sorts of gifts are unappreciated, it's just not practical. In that moment you are diagnosed you need lots of practical stuff. Sick is expensive! I just kept writing down everything I needed and a friend put it all together in a box and I said 'this is exactly what I need!'."

Shelli's kits sell for $150 and she tries to keep costs down but it's hard – "so the more help the better!". The business also keeps her mind off her cancer.

For the moment, Shelli is taking everything day by day and is hopeful that one day, her incurable cancer will be a chronic disease that can be managed.

“If I can get five more years that’s great, if I can get ten it’s awesome and if I get more than that it’s a miracle.

“The best time of the morning is when I wake up and I am still breathing.”

Whatever her future holds, she takes comfort that The KIT Foundation is being built to outlive her.

“We have an amazing advisory board and people just want to be involved. We are without a doubt, the best and the most awesome foundation to be involved with in the world! So join us!,” she laughs.

“The KIT Foundation is here to look after the patient in the here and NOW. The minute you are told ‘You have cancer’, that’s when we step in and start looking after you. We are here for the good times and the bad. This is real and raw and this is what happens. You don’t get away with no tears, trust me.”

‘Kits for Cancer’ come packaged in a vintage style suitcase and can be ordered online at www.kitforcancer.com.

Footnote: Shelli Whitehurst spoke to STA in September 2016. We are inspired by her story and look forward to working with Kit for Cancer in supporting patients.

Call to Screen Type 2 Diabetics for Pancreatic Cancer

“I think the biggest revolutions in pancreas cancer treatment are yet to come.”

Dr Lorraine Chantrill has been a medical oncologist for 10 years, with a particular focus on pancreatic cancer “because it is so terrible and there is so much room for improvement”.

She notes that average survival rates for pancreas cancer have improved but remain abysmal (only 7% of patients survive five years post diagnosis) “and we need to do a lot more work in the prevention space”. She is also calling for screening studies of patients who are newly diagnosed with Type 2 diabetes (possibly via ultrasound) to potentially assist early pancreas cancer detection in some patients. While no link between these two diseases has been established, Dr Chantrill says tumours can affect normal insulin production and a surprise diabetes diagnosis may sometimes be an early cancer warning.

What is your rationale for potentially screening patients who are newly diagnosed with Type 2 diabetes?

We know that probably about 25% of pancreas cancer is cigarette smoking related. Maybe up to 10% of cancers might be linked to inherited changes but that leaves two-thirds with no known cause.

What we do know is that there are some associations. There is an *association* – and I say that carefully – between new onset Type 2 diabetes and pancreas cancer. There is a reasonable rationale to do screening of the pancreas gland in people who are diagnosed with Type 2 diabetes who do not have other risk factors for diabetes.

It's quite simple. If you have a tumour in the gland, it may become dysfunctional so you might not be able to produce insulin as efficiently. As an oncologist I hear the story so frequently: 'I was diagnosed with Type 2 diabetes only a year ago' — so it is a very common story. But it just so happens that this is an age group of people where that can happen anyway. The median age for a diagnosis of pancreas cancer is 67 for primary disease and for metastatic disease it is 71 years old. That is also the age people are commonly diagnosed with diabetes. It is very hard to prove this connection, but I think it might be a place to start. Let's face it, we don't have a screening test for pancreatic cancer. At least start a discussion.

Pancreatic cancer continues to have one of the lowest survival rates in oncology. Is there hope on the horizon?

Definitely there is hope – there is much more hope than there used to be. For example, I have a patient that I have now known for almost four years. I understand that this is an exceptional patient. But this patient just happens to have had an amazing response to treatment and she continues to have an excellent response. She says to me, 'If I live long enough there may be a trial for

me with something new'. And she is absolutely right.

Have treatments for pancreas cancer improved in recent years?

It's true to say treatment (for advanced pancreas cancer) has improved enormously in the last three to four years, and part of that has been driven by a reduction in our nihilism towards the disease. (Previously) we weren't going to do biopsies on patients because they were too sick, or we thought the patients were too sick for combination chemotherapy clinical trials, these kinds of ideas. Fortunately, that has changed and there is much more appetite to do things aggressively for people with pancreas cancer. We have also seen more recent trials in the second-line therapy space, which was unheard of in the past. That constitutes a been a revolution in this disease.

I think the biggest advances in pancreas cancer treatment are yet to come. They are to do with selecting out sub-populations of cancers that respond to different treatments.

How many sub-populations of pancreas cancer might there be and what are the treatment benefits of identifying these patients?

There's still a lot of work to be done but I suspect there are more than four sub-types and maybe as many as 10.

I think the future will be about dividing pancreatic cancer into these sub-types and treating them differently. For example, there is a very small sub-type – representing 2% of patients – that have up-regulation of HER2 receptors and these patients may be responsive to anti-HER2 therapy, like *trastuzumab*. But that represents only 2% of cases. So screening and running a trial for only 2% is very difficult and very expensive. Similarly, BRCA mutated pancreatic cancers may respond better to platinum based therapy, but in Australia the percentage of BRCA mutated cancers is only about 4%. We also believe that the KRAS sub-type

makes up about 7% of cases.

What needs to happen to assist next stage research efforts into this disease?

We really need to do innovative trials using patient derived tissue, which we can't be shy about getting. I think there are a number of ways to do this, including a warm autopsy program, which is something we hope to do. That's a way we can get tissue from several sites to which the cancer has spread – or *metastatic* sites, which is very difficult to attain during life. While we have done a major catalogue of primary pancreatic cancers in terms of whole genome sequencing, we know very little about whether metastases are the same or not.

Is immunotherapy playing a role yet in the treatment of pancreas cancer?

We have seen very poor results with immunotherapy so far in pancreas cancer which is very disappointing but maybe there is a sub-type that may respond to immune-based therapy. We have not found that yet, but that is not to say we should not keep looking.

How important is it to encourage new trials in this disease?

I went to a trials meeting recently and there was debate about how hard it is to get tissue samples from people with pancreatic cancer. Doctors were saying biopsies are hard because patients are too sick and because pancreas tumours can be technically difficult to access. For too long health professionals have been saying 'I don't want to put the patient through that, it is too difficult and these people have a short life expectancy. But patients are saying 'you should be asking us'. For example, if we were able to get biopsies after treatment or during treatment, we might be able to work out some of the clues which enable tumours

to respond to chemotherapy. We don't even know those simple answers, because it is very difficult to obtain tissue, especially for those patients for whom treatment is not working.

What inspires your oncology career?

If you talk to most oncologists you don't have to scratch the surface very deeply to find people have a personal connection with cancer. Most people do these days. My mother died of cancer and I was determined to do something in this field because I thought there was so much need. The other thing that appeals to me about oncology is the amount of research being done. I did a science degree and worked in molecular biology before I did medicine. Oncology is the most exciting part of medicine because molecular biology is having a real influence on diagnosis and treatment. My ambition is to be a clinician scientist, literally trying to do research that informs treatment in the clinic. It is a model that has become more acceptable in Australia but it has not been a very common model until now. In US and European public academic institutions it has been pursued for some time.

The way in which our system is set up has hampered this career path, but it is developing now.

Dr Chantrill is based at the Kinghorn Cancer Centre in NSW and is a Director of the Australasian Gastro-Intestinal Trials Group. She spoke with STA in September 2016.

Oral Mucositis – Expert Opinion,

Prof Dorothy Keefe

“Oral mucositis is quite debilitating, because pain in your mouth, or ulceration in your mouth, makes it hard to eat and to swallow.”

“Pretty much every cancer agent can cause some degree of mouth or gut damage. But it all depends on which drugs and what doses you are using. The range of incidence is from as low as 10% for one drug to as high as 60% for another drug. If you are using a high-risk drug or you are using head and neck radiotherapy it certainly goes up towards 100%.

Oral mucositis is quite debilitating, because pain in your mouth, or ulceration in your mouth, makes it hard to eat and to swallow. So, what happens is you get malnutrition and you get pain and both of those have an impact on your quality of life, so it can be a really big deal. There are some patients, particularly in the head and neck cancer area, who have to have naso-gastric feeding during their treatment because of their mucositis.

Head and neck cancer patients and lung cancer patients are particularly prone to

weight loss anyway and the mucositis just adds to that. How much is due to the mucositis is hard to determine, but people can lose 5-10% of their body weight if they are badly affected.

In this era of personalised cancer medicine, we need to look at the tumour AND the patient. It's about holistic care for the patient and that is what people want, ultimately, including the management and prevention of oral mucositis."

- Professor Dorothy Keefe, August 2016.