Brain Tumour Awareness Month 2020: Prof Anna Nowak

Early in her career, Professor Anna Nowak treated a young woman who had just been diagnosed with the most serious type of brain cancer, a glioblastoma multiforme, or GBM. This terrible cancer has a grim prognosis. Most people diagnosed survive an average of just 15 months and the statistics have hardly improved in the past two decades.

But, as Professor Nowak remembers, this bright young woman had a couple of things in her favour – her tumour was in the frontal lobe, which is a relatively accessible section of the brain in terms of surgery. In addition, the molecular structure of her tumour was such that it was more likely to respond to treatment.

Even still, Professor Nowak would never have dared predict the young woman would defy the odds and live into her late twenties.

But fifteen years later, the young woman is a mother in her mid-thirties "with a partner, two children, a thriving career and no recurrence of her glioblastoma".

"The idea that she would be with us almost fifteen years later without a recurrence of her GBM at that point would have been extraordinary," Professor Nowak says now.

“We do have a handful of people who are with us after 10 years. It is never very many, but we do see those extraordinary five to ten-year survivors.”

> But it is not only the survivors that Professor Nowak remembers. Hundreds of patients young and old have made an impact on her life and career, “and they are memorable for different reasons long after they are gone”.

Professor Nowak says the things she loves most about her job are the patient and family interactions and the research.

> ***It is a real privilege seeing how extraordinary people can be, how gracious people and their families are in the face of a life-limiting illness. Those interactions are incredibly rewarding.***

“Secondly, I am extremely committed to research and being able to offer that hope to patients as well.”

Professor Nowak believes that even patients with a grim prognosis can be provided hope.

“Hope for someone with an early cancer might be hoping for a cure. But with advanced brain cancer, it might be hope for freedom from discomfort, it might be hope for longer time with their family. It might be hope for the opportunity to participate in a clinical trial. There is always something for people to hope for,

even when you are not dealing with a curable situation. We must take the time to learn how much information a patient wants and is ready for."

2030 Vision

Professor Nowak predicts that personalised medicine will become increasingly important in brain cancer, with tumours to be sub-divided into molecular types and treated accordingly.

"Research is generally incremental. We get small areas of growth which build on each other. I expect to see more opportunity for tailored medicine as we understand what makes one person's cancer grow based on molecular sequencing. Then, we will be able to personalise treatments that specifically target cancer growth in that individual. We are understanding more all the time about the particular switches that turn on some types of brain cancer."

She believes the Australian Brain Cancer Mission announced in 2017 will be a key driver in changing outcomes.

This "incredibly positive" initiative will support research into brain cancer treatments and provide $124 million in research funding over the next 10 years.

In the long term it aims to defeat brain cancer, but in the interim, it seeks to improve quality of life, provide all patients with the opportunity to join a clinical trial and build better research capacity.

"While the major focus of this decade-long plan is on trials and treatment and research, it will also examine supportive care," Professor Nowak says.

"We have great hope for the future, but we also have to acknowledge that even if we have no treatments to cure people, it is very worthwhile working hard to make the journey easier. Until we have all the answers, that's what we must do."

May 2020.

Brain Tumour Awareness Month 2020: Prof Antonio Di Ieva

Professor Antonio Di Ieva predicts and hopes that brain cancer will be a manageable chronic disease “much like diabetes” by 2030, and patients will have a vastly improved quality of life.

Speaking to Specialised Therapeutics to mark Brain Tumour Awareness Month, the accomplished neurosurgeon, who has performed more than 2000 brain surgeries in the past 15 years, also expects that while the diagnosis of brain cancer appears to be increasing, neurosurgeons will be doing less brain surgery in the next few decades, as personalised therapies move to the forefront of brain cancer treatment.

“I believe it is achievable for us to be able to treat and manage patients with glioblastoma (GBM) as long-term survivors by 2030,” he said. “While it is going to be very difficult for us to say to anyone ‘you are GBM free’, I hope that within the next ten years, we will have therapies that can help patients live with the illness for a long time. I also hope and expect that we will be able to give them a better quality of life than we are able to give them currently.”

And the decades post 2030 could herald even greater advances.

“In the longer term – by 2040 or 2050 – I am certain there will be less surgery required. Although it is the current state-of-the-art, it is primitive to think we can keep cutting away at the brain in the next decades,” Professor Di Ieva says.

“While we will still remove benign tumours, pituitary tumours, meningiomas, I am certain that cancers like glioblastoma will be more commonly treated with advanced chemotherapies, or genetic therapy or immunotherapy with a specific vaccine. As neurosurgeons we will do less from a surgical point of view, but more in terms of patients’ management and research.”

The past decades have been relatively stagnant in terms of improving survival outcomes for GBM patients, but Professor Di Ieva believes “quality of life has improved a lot”.

“Over the next five years we can expect to see even greater improvements.

“What is important is ensuring that patients can continue doing normal activities – things like staying at work, keeping up with their hobbies and continuing to drive.

“We want to help people remain independent for as long as possible. This is an achievable goal.”

All progress is backed by solid research and for Professor Di Ieva, studying the brain is endlessly fascinating, both scientific and philosophically.

He is firmly positioned at the forefront of Australian brain research, establishing the world’s first computational neurosurgery laboratory at Macquarie Health.

Late last year, he was awarded the John Mitchell Crouch Fellowship by the Royal Australasian College of Surgeons (RACS), the premiere surgical research award of the RACS, as well as a $1,015,000 Australian Research Council (ARC) Future Fellowship to enable the creation of new artificial intelligence tools that will improve standard brain imaging, tumour detection and classification.

“Improved artificial intelligence will be a key feature moving forward – these are the tools that will assist us to diagnose better, to understand more and use this information to improve outcomes,” he says. Now Prof Di Ieva is supporting the

creation of the “augmented” multidisciplinary team (MDT) of the future, where MDT-related decision-making can be enhanced by means of the use of the machine, as emphasised in his recent publication on The Lancet.

“It is through research we make real progress and brain cancer patients should know we are working tirelessly to make a difference.”

Professor Antonio Di Ieva is a full-time consultant neurosurgeon at Macquarie Neurosurgery / Macquarie University Hospital, Associate Professor at Macquarie University, full professor of Neurosurgery in Italy, Associate Professor of Neuroanatomy in Austria and head of the Computational NeuroSurgery (CNS) Lab at Macquarie University. He spoke with ST in May 2020.

New Early Breast Cancer Drug Available Now in Singapore

Singapore, 23 April 2020: A NEW breast cancer drug shown to significantly reduce the risk of cancer recurrence is now commercially available to Singapore patients.

The drug, NERLYNX (neratinib), is an oral medication taken by women with breast cancer who have had surgery, chemotherapy and prior trastuzumab-based therapy.

It has been shown to significantly reduce the ongoing risk of recurrence in HER2+ early breast cancer patients,2 with the greatest benefit seen in women who are also hormone-receptor positive (HR+) and who commence therapy within 12 months of completing trastuzumab-based therapy. For these women, the five-year risk of recurrence is reduced by up to 42%.[1]

NERLYNX is being made available in the region by independent pharmaceutical company, Specialised Therapeutics Asia (STA) under an exclusive sub-license agreement with Puma Biotechnology, Inc.

A number of patients in Singapore have already been treated with NERLYNX since it was made available via a named patient access program prior to regulatory approval.

Dr Yap Yoon Sim, medical oncologist at the National Cancer Centre, who was an investigator in the ExteNET trial which led to the approval of NERLYNX, said the introduction of NERLYNX provided breast cancer patients with a new option to further reduce their risk of recurrence.

“Certain patients with HER2+ breast cancer may still have a significant risk of relapse, even after being treated with standard chemotherapy and trastuzumab-based therapy,” Dr Yap said.

“This risk can vary from less than 10% to more than 30% during the first five years, depending on the size of the tumour and the number of lymph nodes affected.

“We know the risk of recurrence continues even five years post-diagnosis, especially in patients with hormone-receptor positive breast cancer.

“NERLYNX may now provide additional benefit in terms of reducing this risk of relapse, particularly to women with high-risk disease.

“Essentially it gives patients another opportunity to remain disease-free.”

STA Chief Executive Officer Mr Carlo Montagner said oncologists had welcomed the introduction and availability of NERLYNX, with more than 1600 women in Singapore diagnosed with breast cancer every year.

“We are pleased to be able to make this important therapy available to women in Singapore and further expect to ensure its availability in other parts of South-East Asia, including Malaysia and Brunei,” he said.

Singapore health data shows that breast cancer is the most common cancer that affects women in the country, accounting for almost 30% of all cancer cases. It is estimated that one in 15 women will be diagnosed with breast cancer before age 75.[3]

About NERLYNX[4]

NERLYNX (neratinib) is an irreversible tyrosine kinase inhibitor that blocks signal transduction through the epidermal growth factor receptors, HER1, HER2 and HER4.

NERLYNX is the first HER2-targeted medication approved by the Australian TGA, the US Food and Drug Administration (FDA)4 and the European Medicines Agency (EMA)[5] as extended adjuvant treatment for early-stage HER2-positive (HER2+) breast cancer, for patients who have previously been treated with trastuzumab following surgery (i.e., adjuvant trastuzumab-based therapy).

Extended adjuvant therapy is the next step of treatment that follows adjuvant therapy (treatment after surgery) to further reduce the risk of breast cancer returning.

NERLYNX is an oral tablet and works by binding to multiple receptors inside the cancer cell, blocking signals that tell cancer cells to grow and multiply.

About HER2+ Breast Cancer

Approximately 20% to 25% of breast cancer tumours over-express the HER2 protein. HER2+ breast cancer is often more aggressive than other types of breast cancer, increasing the risk of disease progression and death. Although research has shown that trastuzumab can reduce the risk of early-stage HER2-positive breast cancer returning after surgery, up to 24% of patients treated with trastuzumab experience recurrence.[6]

About the ExteNET Study[2,7]

The ExteNET trial was a double-blind, placebo-controlled, Phase III trial of neratinib versus placebo after adjuvant treatment with trastuzumab (Herceptin) in patients with early-stage HER2-positive breast cancer.

The ExteNET trial randomised 2,840 patients in 41 countries with early-stage HER2-positive breast cancer who had undergone surgery and adjuvant treatment with trastuzumab. After completion of adjuvant treatment with trastuzumab, patients were randomised to receive neratinib or placebo for a period of one year. Patients were then followed for recurrent disease, ductal carcinoma in situ (DCIS), or death for a period of five years after randomisation.

The primary endpoint of the trial was invasive disease free survival (iDFS). The trial demonstrated that after a median follow up of 5.2 years, treatment with neratinib resulted in a 27% reduction of risk of invasive disease recurrence or death versus placebo (hazard ratio = 0.73, p = 0.008). The 5-year iDFS rate for the neratinib arm was 90.2% and the 5-year iDFS rate for the placebo arm was 87.7%.[7]

An additional five-year sub-group analysis demonstrated a 42% risk reduction in

women who were HR+ and who had commenced neratinib therapy within 12 months of completing treatment with trastuzumab.[7]

The most common adverse reactions (≥ 5%) were diarrhoea, nausea, abdominal pain, fatigue, vomiting, rash, stomatitis, decreased appetite, muscle spasms, dyspepsia, AST or ALT increase, nail disorder, dry skin, abdominal distention, epistaxis, weight decreased and urinary tract infection.[2]

Puma is conducting a Phase 2 CONTROL study investigating various prophylactic anti-diarrhoeal regimens for the first 1-2 cycles of neratinib therapy. Emerging data suggest that prophylactic management reduces the incidence, severity and duration of neratinib-associated diarrhoea as compared with events observed in ExteNET.

About Specialised Therapeutics Asia

Headquartered in Singapore, Specialised Therapeutics Asia Pte Ltd (STA) is an international biopharmaceutical company established to commercialise new therapies and technologies throughout South East Asia, as well as in Australia and New Zealand. STA and its regional affiliates collaborate with leading global pharmaceutical and diagnostic companies to bring novel, innovative and life-changing healthcare solutions to patients affected by a range of diseases. Its mission is to provide therapies where there is an unmet need. The company's broad therapeutic portfolio currently includes novel agents in oncology, haematology, neurology, ophthalmology and supportive care.

Additional information can be found at www.stbiopharma.com

About Puma Biotechnology

Puma Biotechnology, Inc. is a biopharmaceutical company with a focus on the development and commercialization of innovative products to enhance cancer care. The Company in-licenses the global development and commercialization

rights to PB272 (neratinib, oral), PB272 (neratinib, intravenous) and PB357. Neratinib, oral was approved by the U.S. Food and Drug Administration in 2017 for the extended adjuvant treatment of adult patients with early stage HER2-overexpressed/amplified breast cancer, following adjuvant trastuzumab-based therapy, and is marketed in the United States as NERLYNX® (neratinib) tablets. In February 2020, NERLYNX was also approved by the FDA in combination with capecitabine for the treatment of adult patients with advanced or metastatic HER2-positive breast cancer who have received two or more prior anti-HER2-based regimens in the metastatic setting. NERLYNX was granted marketing authorization by the European Commission in 2018 for the extended adjuvant treatment of adult patients with early stage hormone receptor positive HER2-overexpressed/amplified breast cancer and who are less than one year from completion of prior adjuvant trastuzumab-based therapy. NERLYNX is a registered trademark of Puma Biotechnology, Inc.

- • NERLYNX® (neratinib) **now commercially available** in Singapore for HER2+ breast cancer patients following adjuvant trastuzumab-based therapy
- • Five-year follow up data show NERLYNX reduces risk of invasive disease recurrence by 42% in women with early-stage, HER2+/HR+ breast cancer and who commence therapy within 12 months of completing trastuzumab-based therapy[1]

Further Inquiries

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Pharma in Focus: 16 April, 2020

PHARMA IN FOCUS

By Megan Brodie 16 April 2020

PBAC fees too high for little guy

The owner of Australia's largest independent pharmaceutical company says changes to PBAC fees mean small companies need to budget almost $2 million to make a submission for a medicine to be listed on the PBS, with no guarantee of success.

In a submission on new fees being introduced as part of PBS process improvements, Specialised Therapeutics Australia CEO Carlo Montagner said the proposed fee hikes due to take effect on 1 July presented "a major barrier" to PBS

access for small, independent pharma companies like STA.

“These fee increases will mean the cost of submitting a major submission is now well in excess of $300,000 – irrespective of whether the application is successful,” Montagner said.

“STA has estimated that the combination of fee increases, new fees for various processes and internal costs of submission preparation will mean that the real cost per submission is approaching $750,000.

“Considering that it typically takes several submissions to achieve a PBS listing, companies need to budget almost $2 million for a
single submission, with no predictability that the submission will be successful or commercially feasible if onerous listing conditions are mandated by the PBAC.”

Montagner argues large, multinational companies are more able to bear the upfront cost of larger fees while for smaller companies, they “potentially mean the financial risk is simply too great, especially when the outcome of a PBAC submission is highly unpredictable”.

The STA submission proposes companies like STA with annual revenues of less than $50 million be granted an exemption from paying new fees ‘upfront’ for at least the first two PBAC applications, instead paying back the cost in instalments after a successful PBS listing and earnings of more than $3 million a year.

Montagner says STA’s experience was that demonstrating statistically significantly improved survival data and furnishing positive funding recommendations from key overseas agencies did not guarantee success at PBAC.

In the past year, STA has twice submitted unsuccessfully for breast cancer drug Nerlynx and also twice for myeloma therapy Aplidin at a combined fee cost of almost $1 million. The outcome of its second Aplidin submission will be released next week.

He said the proposed fee hikes, such as the $238,230 fee for the facilitated resolution pathway and the $72,000 cost of an associated facilitated workshop, “appear exorbitant”and “seem disproportionate to the work input required by the Department of Health”.

STA supported a call by Medicines Australia for an independent audit of the

proposed charges, with Montagner saying “more clarity is required”.

Montagner says while “there will always be risk when it comes to bringing new medicines to market”, “the reality is that with the new fees and increases to existing fees, pharmaceutical companies will be spending in excess of $3 million for every drug they try to list”.

“It’s a vast amount of money when there is no definitive predictor of listing success that a company can rely on to determine the degree of investment risk.”

Orphan drugs hardest hit

Montagner says orphan drug submissions will be particularly adversely impacted by the proposed fee hikes as their potential PBS revenue is insufficient to justify the multi-million dollar outlay required to submit them to the PBAC.

“I would like to propose that the first two PBAC submissions for orphan designated drugs are fee exempt, with a further minor submission included (if this is required following an unsuccessful second major submission),” he says.

Montagner says when the full impact of the July 2020 PBAC fee increases is realised in two to three years, small Australian-owned companies like STA “will not be able to take on the financial burden and associated risk to bring these new medicines to Australia”.

“Ultimately, this means that patients will miss out, because the international drug development companies STA partners with to make these therapies available do not have an established presence in this region.

“Of most concern is that Australia will end up like New Zealand, where many companies no longer submit products for regulatory approval due to the low probability of achieving reimbursement.”

Pharma Dispatch: 16 April, 2020

Pharma Dispatch

16 April 2020

STA: New fees a “major barrier” to patient access

Specialised Therapeutics Australia says proposed further increases in PBS submission and listing fees are “prohibitive” for smaller companies and risk becoming a “major barrier” to patient access to new medicines.

The Department of Health recently announced new and higher cost recovery fees for new vaccines and medicines that will be implemented from mid-2020. They build on the range of fee changes implemented from mid-2019.

Specialised Therapeutics Australia (STA) is a privately owned pharmaceutical company led by Carlo Montagner.

“Our mission has always been to fulfil unmet medical needs – we do not in-license ‘me-too’ therapies where there is a comparable competitor already in the market,” said the company in its submission on the proposed fees.

“All products in our portfolio are carefully and prudently selected for the incremental clinical benefit they provide, particularly to smaller patient populations. Typically, we partner with smaller European or USbased biotech companies that do not have a presence in our region. Therefore, if STA did not partner
with these companies, their products would not be available to patients in Australia,” it said.

The company said it backs the concerns raised by Medicines Australia.

“These fee increases will mean the cost of submitting a major submission is now well in excess of $300,000 – irrespective of whether the application is successful,”

it said.

“STA has estimated that the combination of fee increases, new fees for various processes and internal costs of submission preparation will mean that the real cost per submission is approaching $750,000.

“Considering that it typically takes several submissions to achieve a PBS listing, companies need to budget almost $2 million for a single submission, with no predictability that the submission will be successful or commercially feasible, if onerous listing conditions are mandated by the PBAC.”

It said the cost of making a submission is “increasingly prohibitive” but that they present a “major barrier” for independent and privately-owned companies like STA.

“While these commercial considerations are matters for all pharmaceutical companies, larger multinational companies have far greater financial resources to bear this cost upfront,” it said.

It continued, “For smaller companies in this industry with a turnover of less than $50 million annually, these increased costs will potentially mean the financial risk is simply too great, especially when the outcome of a PBAC submission is highly unpredictable.”

The company highlighted its “own experience with recent major submissions” where it said high-level of evidence and improved outcomes for patients had still resulted in rejections.

It pointed to its submissions on breast cancer therapy NERLYNX (neratinib) and myeloma therapy APLIDIN (plitidepsin).

“Even based on the older fee structure and levels, these four applications have cost our company almost $1 million in fees,” it said, adding the new fee structure means pharmaceutical companies will be spending in excess of $3 million for every medicine they try to list on the PBS.

STA backed an independent audit of the changes and proposed special consideration for companies with annual revenues of less than $50 million.

“I am respectfully requesting that smaller companies with revenue <$50M

annually be granted an exemption from paying new fees ‘upfront’ for at least the first two applications, and when, or if, a drug is listed on the PBS, the company then pays those fees in arrears, in instalments when PBS expense on that drug exceeds $3M per year.”

On orphan drugs, STA said, “While the PBAC provides an exemption on the initial PBAC submission for drugs that have been orphan-designated, this is not the case for subsequent submissions.

“As stated earlier, it typically takes two to three submissions for a drug to receive a positive PBAC approval.

“Given this statistic, we are now faced with a real barrier for orphan drugs to be PBS listed as the likelihood of success in the only fee exempt round (first submission) is low, and the revenue that would be generated by the orphan drug insufficient to justify the multi-million dollar outlay required for subsequent submissions.”

The company proposed that the first two PBAC submissions for orphan designated drugs should be fee exempt with a further minor submission included.

PBS Process Improvements Submission

By Carlo Montagner, CEO Specialised Therapeutics Australia

Dear PBS Improvements Section,

My name is Carlo Montagner and I am the Chief Executive Officer and co-founder of Australia’s largest independent pharmaceutical company, Specialised Therapeutics Australia (STA).

We are a wholly family-owned Australian company, supplying specialist therapies and technologies to patients throughout Australia, as well as in New Zealand and South-East Asia. Our interests are heavily focused in oncology and haematology, although we are not confined to these areas. Our mission has always been to fulfil unmet medical needs – we do not in-license 'me-too' therapies where there is a comparable competitor already in the market. All products in our portfolio are carefully and prudently selected for the incremental clinical benefit they provide, particularly to smaller patient populations. Typically, we partner with smaller European or US-based biotech companies that do not have a presence in our region. Therefore, if STA did not partner with these companies, their products would not be available to patients in Australia.

I would like to address two areas of fundamental concern that may impact our business model and ongoing ability to bring these unique medicines to Australia.

1. PBAC new fees and substantial increases to existing fees;
2. Orphan drug applications that are not fee exempt following an initial application rejection

PBAC Submission Fees – The Introduction of New Fees and Increases to Existing Fees

While we wholeheartedly support concerns raised by Medicines Australia (MA) in its submission, I further note that these very substantial fee increases, combined with the introduction of new fees, **are particularly prohibitive for small, independent pharmaceutical companies**.

These fee increases will mean the cost of submitting a major submission is now well in excess of $300,000 – irrespective of whether the application is successful.

STA has estimated that the combination of fee increases, new fees for various processes and internal costs of submission preparation will mean that the real cost per submission is approaching $750,000.

Considering that it typically takes several submissions to achieve a PBS listing, companies need to budget almost $2 million for a single submission, with no predictability that the submission will be successful or commercially feasible, if onerous listing conditions are mandated by the PBAC.

As discussed, this makes the cost of lodging a submission increasingly prohibitive. But for small, independent privately-owned companies like our own, these charges present a major barrier.

While these commercial considerations are matters for all pharmaceutical companies, larger multi-national companies have far greater financial resources to bear this cost upfront.

For smaller companies in this industry with a turnover of less than $50 million annually, these increased costs will potentially mean the financial risk is simply too great, especially when the outcome of a PBAC submission is highly unpredictable.

I acknowledge that there is never a guarantee of success for any pharmaceutical company when it submits to the PBAC for reimbursement. I understand there is not an unlimited pool of funding from the government, and also that not every therapy deserves reimbursement.

It is now apparent from our own experience with recent major submissions, that even when a company has attained high-level trial evidence showing a drug has achieved its primary and secondary endpoints, has demonstrated statistically significant improved survival data, and when the same therapy has achieved positive reimbursement recommendations from key agencies such as NICE, it can still be rejected multiple times by the PBAC.

I would further note that as MA has advised, these PBS submission cost increases seem disproportionate to the work input required by the Department of Health.

MA is calling for an independent audit of the changes proposed in the draft Cost Recovery Implementation Statement (CRIS). STA is supportive of this stance, and agrees more clarity is required – particularly around the new charges proposed for the facilitated resolution pathway ($238,230) and the associated facilitated workshop with one or more PBAC members (approximately $72,000). These charges appear exorbitant and it is not clear how additional funds will be used.

Independent Experience

My concerns stem from personal experience. In the past year, our company has submitted two applications for a TGA-approved breast cancer drug NERLYNX

(neratinib) to be PBS listed for the benefit of all appropriate patients. We have also submitted two further applications for a novel myeloma therapy APLIDIN (plitidepsin) that is providing hope to patients who have relapsed after earlier lines of treatment. Even based on the older fee structure and levels, these four applications have cost our company almost $1 million in fees.

This amount does not take into account all the other necessary costs involved in preparing a detailed submission dossier, including advisory board meetings, market surveys to determine treatment algorithms, and developing a health technology economic model to determine cost-effectiveness outcomes. These items add several hundred thousand dollars to the standard submission costs.

On at least three of these occasions submitting for NERLYNX and APLIDIN, ST was unsuccessful. An outcome for the latest APLIDIN submission is not yet public.

Both of these therapies are approved by highly regarded regulatory agencies, and in the case of NERLYNX, reimbursed in other parts of the world.

I accept there will always be risk when it comes to bringing new medicines to market.

History shows it will typically take two, or even three PBAC submissions to achieve a listing, even with the best evidence available. Given this, the reality is that with the new fees and increases to existing fees, pharmaceutical companies will be spending in excess of $3 million for every drug they try to list. It's a vast amount of money when there is no definitive predictor of listing success that a company can rely on to determine the degree of investment risk. As stated earlier, while large multi-national pharma companies may be able to bear this cost and risk, smaller companies such as STA cannot manage this level of 'upfront' payment combined with the high risk of rejection due to the poor predictability of listing success.

A Potential Solution

A potential solution to this situation is to provide special consideration to pharmaceutical companies that are generating annual revenues of less than $50 million.

I am respectfully requesting that smaller companies with revenue <$50M

annually be granted an exemption from paying new fees ‘upfront’ for at least the first two applications, and when, or if, a drug is listed on the PBS, the company then pays those fees in arrears, in instalments when PBS expense on that drug exceeds $3M per year.

Orphan Drugs

The situation is even more difficult with orphan drugs – that is, therapies that treat people with rare diseases and where there is a high unmet clinical need.

These patient populations are frequently denied effective targeted therapies but have the same right to receive precision medicines that may significantly improve their outcomes.

While the PBAC provides an exemption on the initial PBAC submission for drugs that have been orphan-designated, this is not the case for subsequent submissions.

As stated earlier, it typically takes two to three submissions for a drug to receive a positive PBAC approval.

Given this statistic, we are now faced with a real barrier for orphan drugs to be PBS listed as the likelihood of success in the only fee exempt round (first submission) is low, and the revenue that would be generated by the orphan drug insufficient to justify the multi-million dollar outlay required for subsequent submissions.

I would like to propose that the first two PBAC submissions for orphan designated drugs are fee exempt, with a further minor submission included (if this required following an unsuccessful second major submission).

Finally

The PBS was established more than 50 years ago to ensure that all Australian residents have affordable and reliable access to a wide range of necessary medicines.

I fear that when the full impact of the July 2020 PBAC fee increases is realised in the next two to three years, small Australian-owned companies like ours will not be able to take on the financial burden and associated risk to bring these new medicines to Australia.

Ultimately, this means that patients will miss out, because the international drug development companies STA partners with to make these therapies available do not have an established presence in this region.

Of most concern is that Australia will end up like New Zealand, where many companies no longer submit products for regulatory approval due to the low probability of achieving reimbursement.

The Federal Government may believe that if a drug is important enough, then it will be developed by a large multi-national pharmaceutical company with an established local presence.

In reality, many of the smaller-volume targeted therapies – resulting from the evolution of precision medicine research – are developed by small biotech firms. Many of these firms have great expertise in drug development but are developing niche medicines not typically commercially attractive to big pharma. The STA business model has always been to partner with these companies and fulfil unmet medical needs – in large and small patient populations.

Without the support of smaller companies like STA, valuable therapies being developed by small biotechs may never reach the people for whom they were developed, and where they can provide benefit.

Thank you for considering our submission and we look forward to your feedback.

Company Update – Apr 2020

See below, ST Asia's Company Update / Annual Report for 2018/19. To view this publication, click on the cover image below.

Introducing BREAST

Thousands of Australian women are affected by breast cancer every year. ST is committed to providing new therapies that can make a difference, but we also want to share the stories and experiences of those women who are diagnosed, as well as insights from some of Australia's most respected oncologists. We've produced a breast cancer publication, designed to cover many elements of the experience and answer some of the questions women ask.

Here's a short preview of BREAST. We will be publishing thousands of copies in the next few months, which our team will distribute to oncologists for their patients. We look forward to your feedback.

COVID-19

Specialised Therapeutics CEO Carlo Montagner has appeared on a national business program, discussing the potential impact of the COVID-19 crisis on the global pharma supply chain, and the race for an effective COVID-19 vaccine and

therapy.

He told business journalist Ahron Young that ST's Spanish partner PharmaMar is now fast-tracking trials of its myeloma compound as an anti-COVID-19 agent, with laboratory tests delivering highly promising results. Mr Montanger says Australian pharmaceutical companies have sufficient inventories for the next six to nine months, warning that logistics and supply deliveries must be considered essential services. Click on the video banner above for more.

The Courier Mail: 22 February, 2020

THE COURIER MAIL

By Sue Dunlevy 22 February 2020

Breast cancer test fail

Government denies funding to help avoid chemo

A $5000 test that can indicate whether a breast-cancer patient needs chemotherapy has been rejected for a government subsidy even though it could potentially save thousands of women from having to undergo the harrowing cancer treatment.

The Federal Government's Medical Services Advisory Committee said there was not enough evidence to support a Medicare rebate for the test.

But breast cancer support groups are furious with the decision.

And US breast cancer expert Dr Eric Winer, from the Dana-Farber Cancer Institute in Boston, said he was “shocked” by the MSAC decision.

In the US, all insurers paid for the genetic test for women with HER2-positive breast cancer when the cancer had not spread to the patients’ lymph nodes, he said

“I think it’s a mistake,” he said. “To put it simply for a sizeable group of patients the decision tools you have will continue to be from the year 2000, instead of taking advantage of new tools for treatment decision.”

The Oncotype DX test analyses 21 genes from a breast tumour and can help predict the risk that a woman’s breast cancer may recur, and the likely benefit chemotherapy may have in reducing that risk.

Specialised Therapeutics performs the test in a single laboratory in the US and it has not been approved for use by regulatory authorities such as the US Food and Drug Administration nor by the Australian Therapeutic Goods Administration.

A clinical trial of 10,273 women with breast cancer found nine years after diagnosis the rate of disease-free survival was similar for women with a mid range score in the gene test. Disease-free survival for those who received hormone therapy only was 83 per cent compared with women who received both hormone therapy and chemotherapy (84.3 per cent).

Specialised Therapeutics Australia Pty Ltd had applied for public funding of the Oncotype DX test in Australia but MSAC rejected the application on Thursday night.

A spokeswoman for Breast Cancer Network Australia said they were disappointed the test had been rejected. “We urge the companies supplying these tumour profiling tests to get together with the Government to find a way forward,” BCNA chief executive Kirsten Pilatti said.