Bridging the Gap to Reimbursement for New Therapies

Imagine this scenario: A wealthy Australian woman is diagnosed with early breast cancer. She has her tumour removed but there is some uncertainty as to whether she will benefit from chemotherapy. Her doctor recommends she pays for a breast cancer genomic test that will reveal specific information about her own cancer to help determine whether chemotherapy will make any difference, or whether she can be safely treated with hormone therapy alone.

The test – known as the Oncotype DX® Breast Recurrence Score test – is not reimbursed by the Government, but this patient comfortably pays $5000 to get the result. Like most women who have this test (around 70%), it reveals that she is safe to be treated with hormone therapy alone.

On the other side of town is another woman with the same type of breast cancer. She hasn't got $5000 for an Oncotype DX test, so her doctor decides to err on the side of caution and prescribes chemotherapy, just to be safe. For this woman, it means up to six months of treatment and potentially, debilitating side effects including fatigue, nausea and hair loss. Some effects – like nerve damage – may be permanent. It might be too hard for her to keep working. Her family life and income may be severely impacted.

It doesn't sound fair that two women have the same cancer, but one has a treatment path that is far more gruelling – simply because she could not afford the test that might help her avoid the more onerous path.

Unfortunately, this happens every day in Australia.

I am the CEO and founder of Australia's largest independent pharmaceutical company, Specialised Therapeutics Australia, which provides the Oncotype DX test to Australian women under license from a US partner.

Our company has tried unsuccessfully six times to have this test reimbursed by the government for all Australian women – as it is for early breast cancer patients in many other developed countries – including the US, UK, Canada, Germany, Italy and France. The Oncotype DX test is recommended by the world's and Australia's most

renowned breast cancer specialists as the “preferred” genomic test, because of the strong clinical evidence underpinning it.

There is no question about the Oncotype DX test’s safety, efficacy or utility. Australia’s peak regulatory authority – the Therapeutic Goods Administration – has approved use of this technology in Australia, and every international breast cancer treatment guideline recommends its use. It is prescribed by leading surgeons and breast cancer specialising oncologists every day. This is simply a question of cost. Some might say we should just lower the price, but it’s not that simple. This test is under license from an international partner and already Australian women are offered the lowest price in the world to access the technology. International governments pay more per test for their residents to have it – because they know it offer women with breast cancer an informed choice to avoid 6 months of toxic chemotherapy, and will ultimately save the health budget significant sums in chemotherapy costs.

We realise that the health budget is a finite resource, particularly given the impact the Covid-19 pandemic, and that not everything can be reimbursed by the government.

It is for this reason that Australians, including consumers, industry and governments – must find new ways for Australians to finance access to new innovative therapies and technologies that are not yet reimbursed.

I was still bruised from MSAC’s most recent rejection of Oncotype DX when I found myself in a large retail outlet. Surrounded by signs about interest-free payment plans for everyday items, the idea struck me: If Australians can purchase almost anything for the home via this type of arrangement, why not important health-related purchases that their doctor would like to prescribe, but cannot because they are not yet funded by the government?

With this in mind, we approached third party finance providers – Latitude Finance – to help us find a workable payment solution for the Oncotype DX test and potentially, other prescription healthcare items.

It has taken many months to negotiate, but we are pleased to advise that from March 15 this year, Australian women will be able to undertake an interest-free payment plan over a nominated period of up to two years to more manageably afford this test.

We believe this is a first for the pharmaceutical industry but expect we won't be the last. This will pave the way for other companies to assist patients in this way, because the gap between availability of new therapies and technologies and reimbursement must be bridged without the need for patients to find lump sums that entail tapping into their super or home mortgage equity.

Patients are falling through the gaps as they await affordable access to new treatments or technologies that might improve their prospects, or overall outcome.

While this new finance option is a good short-term outcome for Oncotype DX, the big picture solution must be reimbursement.

STA remains committed to progressing discussions regarding Oncotype DX with the Federal Government to ensure that cost is not an issue for any eligible woman. We know that even a monthly payment plan will render the test inaccessible for some. It is a technology that should be freely and readily accessed and funded by the Federal purse.

The irony here is that reimbursing Oncotype DX will actually save the federal budget and the taxpayer all the associated costs of a woman having chemotherapy treatment – not only the cost of the therapy itself, but potentially time off work and long-term health and economic consequences.

We all recognise that the Federal health budget is not a bottomless pit. But personal health is priceless, and offering more avenues to access these returns control to the patient. Healthcare must be affordable and accessible. Payment plans are one way to help achieve this. If patients, customers and consumers can make an informed decision to spend several thousand dollars to buy a couch or a television and pay later, it's only fair that they can make the same informed decision with their healthcare practitioner to access the latest therapies and medical technologies not yet reimbursed by the government.

Our CEO Discusses the Last Decade and Reveals Future Plans

Watch Video

Paul Cross is a former Federal Government ministerial advisor and senior pharma executive. Since 2012, he has been the publisher and editor-in-chief of three daily digital news mastheads covering policy, funding and politics in the Australian life-sciences sector, PharmaDispatch, BiotechDispatch and HealthDispatch. His independent news services have around 12,000 subscribers. He sat down with Carlo Montagner in August 2018 to hear how STA evolved and what its plans are for the next decade.

Paul Cross comment:

“What is unique about STA is its genesis. I can’t think of any other example of where an Australian pharma company has been privately established and then gone on to successfully commercialise products.

“Of course, you have got Australian companies like CSL. But remember, CSL began as a Government-funded entity that was privatised and then grew a global presence. Has any other company begun with only the backing of a private individual and gone on to do what STA has done?

“From a publishing perspective, what I like about Carlo is that because it is his company, he speaks with great clarity about issues relating to the pharma industry.

“He knows what he wants, when he wants it and how and why the system would benefit. STA is not a listed entity and he’s got all this skin in the game. It makes

him a great advocate for the sector.

STA holds its own among the multi-national pharma companies in Australia because it has a clear voice that comes direct from the CEO. Being independent, Carlo is not beholden to the policy directions from any global head office in New York, or London, or Paris.

“I can remember when ABRAXANE was going through the PBS process. Carlo took a really assertive view. He said, ‘This is what we are going to do, this is why it has to happen’. And it happened. That is what comes from having skin in the game.

“STA is different from the rest. This company is a great example of making things happen.”

August 2018.

CEO Carlo Montagner Discusses Recent Partnership Deal

This article appeared on page 36 in Pharma Asset Insights (a Scrip industry publication). Click on the Fullscreen button in the middle of the magazine below to read it or scroll down to read the article within this webpage.

“Our most recent partnership deal was with US-based Puma Biotechnology (NASDAQ: PBYI).

This novel early breast cancer drug first came to our attention in 2011 when

Puma acquired the rights from Pfizer.

Following a successful FDA ODAC hearing in 2017, we reached out to Puma for an initial exploratory discussion on commercialising NERLYNX in our region. Less than 6 months later, we not only struck an exclusive license agreement , but we have submitted the New Drug Application dossier to the Therapeutic Goods Administration (TGA) and have made NERLYNX available to appropriate Australian patients via a strictly-controlled patient access program using our proprietary access program platform.

We were able to move quickly because, as I am the 100% owner and CEO of the company, our internal review and approval processes are not subject to multiple internal senior management and board reviews. This means decision making and post-deal product commercialisation execution can be rapid.

If we make a commitment to filing a dossier on a particular date — subject to external influences beyond our control — we have always achieved that commitment.

We were looking for a drug that fulfilled an unmet need and provided a reasonable commercial opportunity.

NERLYNX overwhelmingly met these criteria. It is the first FDA-approved drug for extended adjuvant therapy in women with early stage HER2+ breast cancer and is clearly not a 'me-too' product.

In this case, due diligence processes were also expedited. Our team is comprised of senior pharma executives with many years of regulatory and commercialisation experience. With NERLYNX, we were able to rapidly assess the commercial opportunity as well as the likelihood of regulatory and reimbursement success.

Once due diligence was completed, negotiations commenced on the license terms.

Like all our agreements, the Puma deal was tailored to meet the needs of our partner. These arrangements need to be customised as our partners all have different requirements and operate in different jurisdictions.

Making NERLYNX available to women prior to TGA approval has required particular commercialisation skill.

In addition to the usual advisory boards and meeting with key stakeholders, ST also launches early access programs to potential prescribers.

These programs ensure our customers become familiar with the product, but more importantly, they enable appropriate access to patients in need at the earliest opportunity.

Our NERLYNX access program was launched in Australia in late March – four months post-deal.

We have developed a rigorous process for managing these access programs pre and post regulatory approval, and are currently operating several simultaneously.

With NERLYNX, we are targeting a reimbursement approval within 18 months of submitting our regulatory dossier.

Again, we have a strong track record of achieving these critical milestones and now look forward to making this important medicine available to appropriate Australian women."

What I Have Learned Becoming a Pharma Entrepreneur

Carlo Montagner is the Chief Executive Officer and co-founder of Australia's largest independent pharmaceutical company, Specialised Therapeutics Asia. The son of working-class Italian migrants, he and his wife Bozena established the company just over 10 years ago without any institutional investment and by selling accumulated personal assets, following international careers and diverse roles inside some of the world's biggest and most successful pharmaceutical companies across Japan, Europe and in the United States. Buoyed by experience, Carlo and Bozena established Specialised Therapeutics with a single foundation product that was initially rejected, but went on to become one of the most successful chemotherapies ever commercialised in Australia. His company has since built an expansive drug portfolio, employs close to 50 staff with recently established regional headquarters in Singapore. These are his thoughts on becoming a pharmaceutical entrepreneur.

"ONE of our children aspires to eventually succeed me as the CEO of our independent pharmaceutical company. The other night she asked me, 'So Dad, what school do I need to go to and learn how I run the company?'

A straightforward question, but one that got me thinking about whether entrepreneurs really are born or made, and how you school the next generation to

innovate in pharma and to bring an entrepreneurial mindset to a high-risk, yet risk-averse environment.

Born or Made?

With the 20/20 vision afforded by hindsight, I can see that a large part of my own entrepreneurial mindset is ingrained. Yes, there have been many lessons along the way, there have been great mentors, there has been dogged determination, risk, planning, good decisions and what some may call luck or fate. There have been people and lessons along the way that helped achieve the next goal. But an inner drive to build a business and leave a legacy was part of my make-up. I doubt any of my primary school teachers would have predicted that I would one day create the largest privately owned pharmaceutical company in Australia. But I always had an inner desire to make an impact.

In my younger years, I toyed with the idea of pursuing a career in dramatic arts. However I soon realised that my acting ambition exceeded my talent. When a dramatic career appeared unlikely, I began working in a national retail chain of delicatessens to fund my university studies with the plan to become a clinical psychologist following graduation.

I quickly navigated the ranks to management and found myself at a crossroads: should I pursue retail management or stick to my psychology studies?

I loved the cut and thrust of the retail world. It allowed leadership with substantial people and fiscal responsibility, with scope to be creative and entrepreneurial. While the retail world is competitive and innovative, I decided this path long term would not provide the intellectual stimulation I enjoyed studying psychology.

It was during my post graduate studies in child psychology that I began to combine my passion for learning with my entrepreneurial vision.

I developed a plan to open a national network of specialist, education-based childcare centres. Obviously I was not the only one who saw that opportunity, but I was born into a hardworking migrant family who took on tremendous personal risk in emigrating to Australia but once established here were fiscally risk averse

to investing in anything other than bank term deposits and the family home. I had developed a similar mindset. Without the initial capital (and willingness or even basic awareness to invest in high risk capital raising activities) I could not bring these plans to fruition.
Instead with my girlfriend of the time, and now-wife Bozena Zembrzuski, we bought land (a very safe traditional investment!), before heavily researching and undertaking feasibility studies into establishing an export-centred snail farm on Melbourne's outskirts.

As a result of this research we had the confidence to invest our time and spare funds into what was, as the local newspaper reported at the time, quite a bizarre venture.

Somewhat ironically given my impending pharmaceutical career, we shelved these plans because of an extremely complicated bureaucratic landscape. There were simply too many local council and regulatory barriers in our path. While frustrated I could not build my own business at the time, I knew deep down that I wanted my own enterprise. It was about finding the right fit, at the right time.

It's a Marathon, not a Sprint

Any successful entrepreneur needs to begin with the right idea and a crystal clear vision of the targeted outcome.

To back this up, you need heavy research, sound financials and ultimately, a determination to push through inevitable barriers. When I finished my post-graduate degrees, I embarked on a role in the pharmaceutical industry, confident it would ground me in the corporate world, stretch me intellectually and meet my need to be entrepreneurial.

I set about building a solid foundation of pharmaceutical business learning. Unbeknownst to me at the time, this mission would take years. Along the way, I was offered several promotions that would improve my managerial status and salary.

I turned down several of these opportunities. Each time, despite the prospect of attractive pay increases and the recognition that comes with promotion, I knew I

had not truly achieved my current role targets.

Since my vision was to succeed across all key elements of the pharma business before I stepped into more senior roles, I decided not to move to other roles prematurely.

So, I worked with over-the-counter (OTC) products, launched a range of vitamins into supermarkets, managed mature primary care prescription products and launched several specialty hospital products. I climbed the ladder carefully, strategically and prudently. My advice to aspiring entrepreneurs? Lay the right foundation. Work internationally and gain an intimate understanding of the complex regulatory and commercial environments globally, and cross-culturally. Remember, this is a global business. It is vital pharma leaders understand all elements and intricacies of the pharma business and demonstrate tangible success in any role before taking the next step.

Manage Risk with Experience

After many years working in the pharma industry globally, one learns to cope with the many inherent risks posed developing and commercialising medicines. It is a business where more than nine out of 10 drugs typically fail to reach the market. Once in the market, medicines can still fail, due to pricing pressures or poor prescriber uptake. So we are building a business around products that have a high probability of failure, and a very small probability of meaningful success.

In my experience, it is about accepting, understanding and qualifying the risk that is evident at every stage of drug commercialisation and development.

Even if all clinical trial endpoints are met, there is no guarantee of regulatory and/or commercial success.
And when a drug is on the market – it's jumped through all appropriate regulatory hoops and been given the government tick of approval – this risk remains. A series of unexpected adverse events can quickly change how widely a product is prescribed. Many will remember what happened with Vioxx.

You have to account for factors that might be outside your control and mitigate the impact of potential market competitors.

I am emboldened by the depth and breadth of experience in this sector. If I was in the tech industry, it would be advantageous to be younger where regulatory hurdles are few and far between. But in this business, having more than 20 years' experience enables a full analysis of the potential pitfalls and factors required for commercial success.

This process ultimately enables a balanced, risk-mitigated decision. A good example of this was our decision to continue with the commercialisation of our treatment Iclusig. This highly effective therapy is prescribed for Chronic Myeloid Leukaemia and is the only treatment that works in patients with a particular genetic mutation.

Soon after launching in the US, patients reported experiencing more side effects than expected. The FDA suspended its approval and Iclusig was then (temporarily) withdrawn from the market.

There was a risk of failure if we continued with our application (for Australia). But I decided to proceed with our regulatory and reimbursement processes as I strongly believed patient benefits outweighed the risks observed in the US.

We also openly discussed with our regulators here in Australia how best we could avoid a repeat of the US experience.

Today, Iclusig has been successfully and responsibly prescribed to many patients with this disease.

Take the Leap

For many of our years working in pharma, Bozena and I often discussed how we "could do it better'. Our view was that pharma companies in general were becoming too risk averse, more bureaucratic with a greater focus on ROI rather than patient outcomes. This meant they were seeking to develop the next blockbusters and not paying enough attention to specialist medicines that fulfilled an unmet medical need in patient groups with unique but rarer diseases.

Find Great Mentors

Mentors are vital to young entrepreneurs and I have had several but two stand out.

Pascal Soriot is the global chairman and CEO of Astra Zeneca. He brought me from Australia to the US in a senior role. From him, I learnt the importance of attention to detail and being data driven. He taught me that facts and numbers reveal the 'truth' and should underpin key strategic business decisions. He also instilled a belief that gut instinct alone is not a decision making tool on which to rely.

Pascal also taught me to treat a business as if it was your own.

He taught this by example, when he critically reviewed, sometimes painfully so, all key commercial recommendations by any of his direct reports, regardless of seniority and experience, with the sole objective of ensuring it was the right decision for the organisation. He was not only a key mentor in management skill, but in demonstrating how passion drives sound business decisions.

My other stand-out mentor was US entrepreneur and philanthropist Patrick Soon-Shiong. He is a complex man who invented Abraxane, the drug that eventually became STA's foundation product. When one reads his history from working as an underpaid surgeon in apartheid South Africa, through to becoming a billionaire entrepreneur, there can only be admiration for his ability to persist through adversity against many 'naysayers'.

As a surgeon and entrepreneur, he was a risk taker who persisted when he believed that developing Abraxane would provide a solution to a problem others could not see. He did this despite seemingly more experienced or more knowledgeable people advising him that his idea would amount to nothing. Ultimately, he taught me to be persistent and not to let negative voices cloud your vision.

Stick to your Knitting

"Sticking to your knitting" is an old but valuable adage and in my mind, it is

essential to business success.

You must stick to what you know. In my case, it's pharmaceuticals. If, after all these years, I cannot successfully commercialise a drug, then there's something wrong with me!

I can also see that when entrepreneurs diversify too quickly that they start making bad decisions. Look at (Australian businessman) Alan Bond when he bought the Channel 9 media company. He was a successful entrepreneur but knew nothing about running a television network. My view is that ego probably got in the way, as did the need to expand too quickly. He ended up selling the company back to Kerry Packer for reportedly less than half the original sum. So the message here is, don't let ego take over and don't expand too quickly. Stick to what you know, continually evolve and refine your skills. Only then can you execute with confidence.

Building a Pharmaceutical Company from the Ground Up

When I started Specialised Therapeutics, I understood the principles of making a pharma drug successful, but I did not understand the 'nuts and bolts' of putting a business together.

This was an enormous challenge. When you work in big pharma, it is a well-oiled machine and the groundwork is well established. We had to live and learn. No-one is born knowing everything and there is no handbook to starting and running a pharmaceutical company, because it is such a unique business. We learned our business lessons along the way, sometimes the hard way. Even the hard lessons have enriched our experience. I am proud of what we have developed and it was what we had in mind all along: we wanted an agile commercial business with a team that was able to make decisions and bring products to market quickly. We don't have a lot of the red tape that exists in larger pharma companies. This means we can get products to market and patients quickly and as seamlessly as possible.

A CEO’s Biggest Challenge

The biggest challenge is bringing the right people into our company who are aligned not only intellectually, but culturally. As we grow, we are mindful of attracting and retaining the right people with the right qualifications, who have the same sense of urgency and ideals.

From a commercial perspective our challenge is to continue building the portfolio. This is the ‘leaky bucket syndrome’.

You can in-license a great product but as soon as a patent is granted you have a finite window to maximise the commercial opportunity. It is our endeavour to keep filling the leaky bucket to ensure the company keeps evolving and growing. This means staying one step ahead, and being aware of the wider regulatory framework nationally and internationally. You need a global perspective and an awareness of key health demographics and policies.

Look to the Future

When there are difficult times and difficult decisions, it can help to project forward. I think to myself, ‘In 1, 2, 3 years from now I will say, the outcome was well worth the adversity faced and the effort invested’.

I do not have a numbers target for what I want this company to be worth. Bozena and I do not define the success of the company by numbers alone, but rather what it achieves by contributing to society. Sure we need to be profitable, and the more profit we generate, the more we can contribute to society by making available medicines that really make a difference to people’s lives. In turn, this makes our lives more meaningful.
I had a deliberate strategy for building my career, and my goal now is to keep strategically building upon STA’s foundation, and grow organically. A strong and sound corporate ballast will underpin further success.

I have no doubt that we will be a bigger company in the next few years. But I am determined that the same core values behind our early success will shape our future.

I am driven by a need to keep improving and not accept the status quo. Good enough is never good enough.

What does Success Look Like to a Pharma CEO?

Very simply, being successful in pharma is bringing to market a drug that meets the needs of a patient.

You may have the best science in the world, you may bring products to the point of commercialisation, but if they are ultimately rejected by authorities like the Therapeutic Goods Administration and the PBS, you have nothing.

So, finally getting that medicine to the right patient at the right time so it improves real world outcomes for patients and their families, while managing to do this profitably, is the only real measure of success in this business.

Back to the Original Question – Are Entrepreneurs Born or Made?

So returning to the original question of whether entrepreneurs are born or made. My own conclusion is that fundamental entrepreneurial attributes are hard-wired.

However, these innate personal characteristics need to be nurtured by the right mentors at critical development stages in order for potential to be realised.

An entrepreneurial mindset is never a guarantee of success. But the right people and the right learnings combined with that mindset will give that entrepreneur the best chance to reach their full potential.

Carlo Montagner, February 2018

Access Programs and Why We Need to Tell Physicians About New Medicines Available to Patients

When it comes to healthcare, Australians are fortunate.

We have a world-class health care system that includes an amazing government initiated and managed scheme to ensure that new drugs – perhaps already approved internationally — can be made available in this country, with the approval and supervision of treating doctors.

This scheme is known as the Special Access Scheme (SAS) and was introduced by Australia's Therapeutics Goods Administrations (TGA) "in recognition that there are circumstances where patients need access to therapeutic goods that are not on the ARTG". (https://www.tga.gov.au/form/special-access-scheme).

The motive driving the TGA to initiate the SAS scheme is laudable. However, I continue to hear of many cases, specifically in oncology and haematology, where

physicians are completely unaware that special access programs exist.

I recently heard about a patient with a life-threatening illness who sold his home to import and fund access to a new, innovative and expensive cancer therapy that was not yet approved for use in Australia.

But unbeknownst to both the patient and his physician, there was a special access program – fully compliant with Australia's laws – that would have enabled him access to this unapproved medicine with significantly less financial sacrifice.

Further, this access program was initiated by the pharmaceutical company that developed, manufactured and ultimately imported the drug into Australia for the specific purpose of enabling Australians access to this cancer therapy prior to its regulatory approval.

And herein lies one of the great dilemmas facing pharmaceutical companies developing specialty medicines.

Under the Medicines Australia Code of Conduct, proactively communicating information to a medical professional about the availability of any unapproved drug via an access program under the auspices of the TGA SAS is forbidden, as it is seen to be advertising or promoting an unapproved specialist medicine.

This is where I say – and many of my medical oncologist and haematologist colleagues agree– that there has to be a rethink.

MA must acknowledge that there is a significant difference between advertising and informing.

At the very least, we must have absolute confidence in the decision making process our health care professionals undertake when considering prescribing an unapproved therapy.

These decisions are being made by highly educated, intelligent professionals who have dedicated their lives to medicine. These doctors are not going to provide a medicine to a patient just because they have heard about an access program. They will research information to make the right decision, for the right patient, at the right time.

Given the tremendous workloads of specialist physicians, it is unreasonable to

expect that they would be fully aware of all available access programs. And there are many, as numerous innovative therapies have emerged in recent years. My company alone has several access programs in place.

The pharmaceutical industry should be encouraged to ensure all appropriate physicians are made aware of any access programs responsibly, and without making any promotional claims about the efficacy and safety of these medicines.

This information will enable physicians and patients to make informed and timely decisions about whether they wish to access the unapproved drug.

More broadly, patient and public health lobbyists are also calling for access program information to be available via a central national database, so physicians and patients are aware which new therapies might be available, albeit with special provisions.

Such a database would ensure that the intent of the TGA's SAS is fully realised, ensuring all Australians have the opportunity to access innovative but unapproved new medicines, when deemed appropriate by their physician and when strictly supplied by legitimate medicine manufacturers and developers.

What I want from Pharma: Medical Oncologist Explains

Associate Professor Gary Richardson is the Director of Oncology Clinics Victoria, Director of Cabrini Academic Haematology and Oncology Services and an Associate Professor of Medicine

at Monash University. He previously held the position of Director, Department of Medical Oncology and Clinical Haematology at Monash Medical Centre. He is a Fellow of the Royal Australasian College of Physicians. Specialised Therapeutics sought his insights on medical and pharma interactions.

By Assc. Prof. Gary Richardson, Director of Oncology Clinics Victoria and Oncology Services and Assc. Prof. of Medicine, Monash University

Specialised Therapeutics' new business model can't be a bad thing, because interactions between pharma companies and the medical profession have definitely changed, thanks to the digital revolution.

In the old days when doctors saw pharmaceutical company representatives, it was all paper. That meant that part of a rep's role was to bring papers, show you evidence, outline details from a recent scientific meeting as well as spend some time selling the drug. Those days have gone, because everything is on the web now. You get the alerts and the data is there. There are not that many practice-changing things that occur on a day to day basis and if there are, you will find out about it straight away.

What do I want when a pharma company representative comes to my office? I want information about new clinical trials and about drug access programs. I also want to know what's in the pipeline, what's coming to Australia and what we can do in the space we operate. I don't really want old information about drugs that are already available – I already know that information. What I would like is early information, that I may not yet have seen or been able to access. That kind of information is really good, but that is not so easy to get all the time.

(Under the Medicines Australia Code of Conduct), pharmaceutical representatives are prohibited from speaking openly and freely about access programs for medicines that are not yet approved for marketing in this country – despite the fact these drugs may be already approved and available overseas.

There needs to be a rethink. It is ridiculous that you can't discuss medicines that are 'off-label' or drugs that are potentially coming in the future. There seems to be a fear you will somehow break the rules. I find it insulting that authorities think you might be swayed by these sorts of discussions.

Consider the landscape around five years ago, when all the immunotherapy trials were being done in melanoma overseas. Abiding by the letter of the law, no-one in Australia could discuss any of those medications with anyone, at all. You understand that a trial is going to come, and these drugs are being used overseas, but you are not allowed to talk about it. It just seems crazy, as not all oncologists attend overseas meetings or has seen an original presentation – particularly if it's not in their own area of expertise.

As a doctor, you should be able to make a decision whether a drug should be able to benefit any particular patient you have. The doctor has to make the decision to benefit the patient, because that's what we do.

In terms of other interactions with pharma, I have no problem with transparency reporting, (where pharmaceutical companies acknowledge payments to individual

doctors for services or contributions towards them attending education activities, including flights and accommodation). But I do think it should be the same across all industries – it should be the same for politicians or lawyers. I am not worried if someone puts my name in the paper and notes that I've been paid (by a pharmaceutical company) to attend a meeting. The doctors that worry about these things the most I think, are the younger doctors. And particularly the ones that are still working in the public system, because it seems to me that they are very wary of pharma.

At the end of the day, working with pharma is a double edged sword. Pharmaceutical companies provide good drugs and sponsor clinical trials. The downside is that the industry as a whole, is largely perceived to be about money and many of the big pharma companies are beholden to shareholders.

What would I say to younger doctors who might be wary? At the end of the day, pharma companies are in the business of creating drugs that work. And there are some really amazing drugs that have been made. There are a lot of positives. And this move to remove financial incentives from reps based on volume of sales achieved takes away that pressure to sell and paves the way for a more open discussion."

*Associate Professor Gary Richardson spoke with Specialised Therapeutics in August 2017.

Guest Blog: Rare Cancers Australia chief Richard Vines discusses cancer drug access

Our company has enjoyed a long-standing relationship with Rare Cancers Australia, supporting this organisation's ongoing endeavour to provide all cancer patients with timely and affordable access to new cancer therapies. We are proud to introduce guest blogger Richard Vines, the CEO and co-founder of RCA, as he passionately but simply explains the need for change and how it can be achieved.

IN MY OPINION

By Richard Vines, Chief Executive Officer and Co-Founder Rare Cancers Australia

Consider this: There are two brothers and both are diagnosed with cancer. One has a rare tumour and one is diagnosed with melanoma. Both go to the same oncologist and both are prescribed the same immunotherapy drug. One brother walks out paying $30 a month because the drug is PBS listed for melanoma, while the other one needs to find $10,000 per month.

Does that pass the 'pub test'? But this scenario gets worse. We know that when a drug is listed on the PBS, the PBS does not pay pharma companies the official retail price because they have huge buying power and they can negotiate the best financial deal. This is normal and acceptable commercial behaviour. The rare cancer patient has already contributed his tax to help the Government pay for that drug's broad accessibility for more common cancers, like melanoma. But then, he has to go and pay full retail price. So, you can see, the inequity just builds and builds.

In my role as CEO of Rare Cancers Australia (RCA), this scenario for patients is heartbreaking, and it is not uncommon. The frustration is palpable, it's ongoing and I am seeing this with our patients every day. There are 240 acknowledged 'rare cancers', impacting thousands of patients in Australia.

These patients inevitably reach a point where they run out of PBS funded treatment options. Then, the affordability factor means they have nowhere to turn, despite the fact that there are often life-saving, or life-extending, medicines available. These patients – tax-paying Australians – are looking at prohibitive costs, of perhaps $6 – 8,000 a month.

The PBS System is one designed to carefully steward taxpayer funds with strict guidelines for evidence and cost-effectiveness. But in reality, the level and quantity of evidence required by the PBS is not attainable for rare and super rare cancers. This means that medicines invariably struggle to get reimbursed for these small patient populations. We need realism and flexibility.

I was talking to a mesothelioma patient the other day. The drug that he wants, or will need as a next step in trying to survive, is going to cost him $10,000 per month. He is about 55 years old and he can trace his disease back to a time when he was working in a factory at about 19 or 20 years old. The possibility of him getting any legal compensation is minimal however, and he wants an immunotherapy drug. We can't get him enrolled in a trial, because the selection criteria is really tight, so what option is he left with? Nothing. Should he re-mortgage his house and leave his family with fewer funds to buy himself some extra time?

There are drugs that are already available in this country and I call these

medicines the ‘low hanging fruit’ in this whole debate. These medicines have been approved by the TGA for at least one common cancer type so we know that they are safe (within reasonable bounds) and that the supply chain has been verified. We also know that they are effective in rare cancers. Let’s find a way to use them, for this mesothelioma patient and all the others.

To fix this we need everyone at the table, not just the Government but also the pharmaceutical industry, the clinical community, public servants and of course, patients and patient advocacy groups like ours.

For a start, the Federal Government needs to take a pragmatic approach. It must acknowledge that it is not always going to have all the evidence it needs to list a medicine for rare indications – it’s just not possible, given the size of the patient populations we are dealing with. We have seen, and applaud, instances where Government authorities demonstrate this kind of flexibility

Take the recent case of Vorinostat. This medicine was TGA approved in 2009 for the treatment of cutaneous manifestations in patients with cutaneous T-cell lymphoma (CTCL) with progressive, persistent or recurrent disease subsequent to prior systemic therapies.

A subsequent PBAC submission was rejected for this rare indication due to ‘unacceptably high and uncertain cost-effectiveness ratios.’

Advising the knock-back, the PBAC noted that the quality of data within the submission was extremely limited, due to small study sizes and heterogeneous, non-comparative data.

In 2016, we (RCA) worked with the company involved to invest in an additional analysis that would support a high quality resubmission.

The PBAC showed its flexibility in assessing this submission (e.g. allowing comparison to palliative care for the cost effectiveness analysis) and then, following successful price negotiations, Vorinostat was finally PBS listed on 1 July 2017.

This was a great outcome and something we, at RCA, are very proud of. Now I believe we must continue seeking new ways of collecting both trial and real world data. To do this, clinical trials especially Government-funded investigator trials

need to have broader and wider inclusion criteria.

We would particularly like to see an allowance made so that 10% of places on all clinical cancer trials are reserved for rare cancer patients. This would not cost much, and could be done in such a way as to not detrimentally affect the main trial outcome, should the rare indications produce lower quality results. Companies and universities could do this tomorrow. Clinical trials are the best, safest and smartest way for cancer patients to access new and experimental therapies.

Government and industry also need to look at how they can make small changes, to ensure it is commercially attractive for industry to go to the effort and expense of applying for drug listings for small populations.

In our recent 'Rare Solutions' report we called for the introduction of multi-indication submissions as a means of allowing companies to apply for rare indications at the same time as common ones – thereby saving some of the inevitable double up that happens when applying for the same drug multiple times. We were very encouraged that the Health Minister announced, at the launch of our report, that he has instructed the chair of the PBAC to begin looking at mechanisms for pan-tumour applications, but we all need to work hard together to make this a reality.

Pharmaceutical companies can't just sit there with medicines on the shelf that might help rare cancer patients and not try to make these drugs available. Companies need to be assertive and get on the front foot. If they have a drug listed for breast cancer, then anything they can add on to that is a bonus. I say to them, 'Do a bit of extra work and open up other indications so that more patients can access the treatment'.

And oncologists need to get active and advocate. At the end of the day, they are the people who have to look a patient in the eye and say, 'I am sorry, there is a drug that can help you but it is going to cost you $10,000 a month'.

Speaking generally, medical professionals are not traditionally political creatures, but when it comes to rare cancers, they need to be. Sometimes these doctors may just need to ruffle a few feathers to get a good outcome for the people whose lives are in their hands.

At the end of the day, who gets to decide a patient's treatment? It should be a patient's oncologist, not an economist. It's time to act.

For more information, please go to www.rarecancers.org.au

When Customers Come First, Not Dangling Carrots

I recently bought a red Tesla. It's a battery operated, engineering marvel that doesn't require petrol, can be recharged via a rooftop solar panel, is sleek, modern and chivalrous to boot – with doors that open automatically on the owner's approach.

While I'm an unapologetic and long standing motor car tragic, what really clinched the deal was the way this beautiful piece of machinery was sold to me.

Tesla does have showrooms in Australia, but you can't actually buy these cars

from a showroom.

When you go to a Tesla showroom, expert staff – obvious car enthusiasts like me – demonstrate and provide all manner of information about these cutting edge vehicles.

Our "sales" conversation was educational, informative and involved a pleasant exchange of information that ultimately, led to me purchasing a Tesla product online. At the showroom, I felt no sales pressure but was provided with enough information to make my own decision.

By being informed and well-educated by the representative, in a 'non-salesy', low pressure environment, I was free to consider the actual merits of the Tesla without the distraction of the typical car sales process. I knew the various Tesla representatives I had spoken to in the showroom were not receiving sales commissions, so the information provided was passionately, factually and legitimately delivered.

I tell this story because, as the CEO of Australia's largest independent pharmaceutical company, I have made the decision that from February 1, 2017 our in field company representatives who call on current and potential prescribers of our therapies will no longer be incentivised by the volume of prescriptions written in their territories.

Instead, financial rewards achieved by our people will be based on other performance measures – like the extent of their product and therapeutic knowledge, their level of customer service and engagement, their commitment and dedication to ensuring the patients who would most benefit from our therapies are given the best chance of accessing them.

Why are we doing this? Because if you motivate frontline representatives with a financial carrot, then it is commonsense that those frontline staff are going to prioritise selling products instead of focusing on the specific needs of the patients the product can treat.

Like Tesla, I want doctors to know that when our field force representatives approach them about our therapies, they can engage in a legitimate and genuine exchange of information that is educational and informative.

I want them to feel comfortable in the knowledge that our representative is not being financially rewarded for ‘shifting more units’.

Conversely, I want our people to be truly engaged and to make customer and patient care paramount. I want them to engage and educate without the pressure of sealing a deal.

I want them to strive to achieve – but not sales targets. Success can be measured in other ways that are still tangible.

This approach does fly in the face of how most pharmaceutical companies in Australia and around the world typically operate.

But I am convinced this is the most transparent approach. Our customers – predominantly oncologists and haematologists – can see through a sales pitch. Most consumers can, in whichever industry you work.

This is not about taking an ‘airy fairy’ approach to sales. Quite the contrary. As an entrepreneur with a strong commercial bent, I care passionately about the business I founded, the pharmaceutical industry and the bottom line. Without profitability, there is no pharmaceutical industry, which is able to underpin breakthrough and life saving therapies and technologies.

I staunchly believe this approach will translate to desirable commercial outcomes, because success begins with a great product that fulfils a marketplace need.

Sales are achieved when customers are educated about a product’s merits and benefits. If you have the right product, then the outcome is assured.

When there is an inherent confidence in a product, there is no need to reiterate and ram home tired sales messages.

Our products are medicines that fulfil unmet medical needs. They are not ‘me-too’ products, but are carefully selected for in-licensing to our regions (Australia, New Zealand and South East Asia) because they are innovative and different. Like the battery operated Tesla car, they are not mainstream, but niche-market. The right people will prescribe them if they have the right information and there is a genuine medical need in the community.

Interestingly, my sales tactic sentiments are being echoed in other industries.

In recent weeks, consumer groups have called on the banking industry to come clean on how staff bonuses really work.

These groups warned that some consumers felt bullied into buying bank products by over-zealous sales people who were chasing their own bonuses, instead of providing real, transparent and legitimate information that might actually improve a customer's financial prospects instead of their own.

The customer should always come first and in the pharmaceutical industry, I would say it is even more important.

Our customers are doctors and ultimately, the patients they care for. Their health is their most prized possession. Our sales should only be made when it's right for them, based on the best information available, imparted by an expert, educated field force.

When the basics are in place, the rest will follow. Just ask Tesla.

This opinion piece was published in the Herald Sun on February 10, 2017

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.

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.