New Drug To Fight Hospital Superbug Infection

Melbourne, Australia , 20 June 2012 – A new therapy to treat a common hospital superbug* infection Clostridium difficile, will soon be available to Australian & New Zealand patients.

Melbourne biopharmaceutical company Specialised Therapeutics Australia Pty, Ltd. (STA) has entered into an exclusive distribution & license agreement with US based Optimer Pharmaceuticals, Inc. (Optimer) to develop and commercialise the drug DIFICID in Australia and New Zealand.

This macrolide antibiotic therapy, taken in tablet form, is regarded as a breakthrough treatment to help fight the serious CDI, which typically develops in patients using broad-spectrum antibiotics. The organism – which is resistant to many common household and commercial disinfecting agents – targets the large intestine, causing diarrhoea. It is extremely common in hospitals and aged care facilities and can be fatal.[1]

A recent media report indicated 14 Victorians died from the infection during a 15-month period in 2010 and 2011.[2] According to data generated by the Quebec provincial hospitalisation database, there were 7004 cases of C. difficile across Quebec from April 1st 2003 to March 31st 2004, and 1270 people died after contracting CDI.[3]

STA Chief Executive Officer Mr Carlo Montagner said: "CDI presents a serious bacterial health threat and current CDI treatment options available in Australia and New Zealand are limited. Our license of DIFICID provides a great opportunity to bring a much-needed new therapy to patients."

DIFICID is the first in a new class of macrolide antibiotics, which are minimally absorbed by the bloodstream and have been shown to fight the CDI infection while leaving healthy gut flora untouched.4 DIFICID works by inhibiting the bacterial enzyme RNA polymerase, resulting in the death of C. difficile bacteria.[4] Patients typically develop CDI when using broad spectrum antibiotics,

which disrupt normal gut flora and enable the infection to take hold.

Hypervirulent strains of CDI, including PCR ribotype 027 strains recently identified in Australia, have been associated with epidemic spread and high rates of severe disease and death.[5]

Risk factors for CDI include exposure to antimicrobial drugs, gastric acid-suppressive therapy, advanced age, prolonged hospitalisation, cancer chemotherapy, co-morbidity and immuno- suppression. Although most cases have been in hospital inpatients, increasing numbers of community-associated cases are now being reported.[1]

A leading Australian authority on C. difficile, Professor Thomas Riley from the University of Western Australia, said data showed patients treated with DIFICID were "significantly less likely" to develop recurrent infections.[6,7]

He said new treatment options like DIFICID were highly desirable, with infection rates rising "two to three fold" in public hospitals around the country.

An application to make DIFICID widely available in Australia has been filed with the Therapeutic Goods Administration, with the drug expected to be launched by June 2013.

Optimer Chief Executive Officer Pedro Lichtinger said he looked forward to DIFICID being widely available in Australia and New Zealand. "We are committed to enabling better outcomes for patients with this difficult to treat infection. I believe, this is a truly innovative therapy providing a new patient option for an unmet medical need." he said.

DIFICID is approved by the U.S. Food and Drug Administration (FDA) for the treatment of Clostridium difficile-associated diarrhoea (CDAD) in adults 18 years of age or older. Likewise, the European Commission granted Marketing Authorisation to fidaxomicin for the treatment of adults with Clostridium difficile infections under the trade name DIFICLIR™.

* Superbug is a common reference to an organism or infection, which is resistant to multiple antibiotics.

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About CDI

Clostridium difficile infection (CDI) has become a significant medical problem in hospitals, long-term care facilities and in the community. CDI is a serious illness resulting from infection of the inner lining of the colon by C. difficile bacteria, which produce toxins that cause inflammation of the colon, severe diarrhoea and, in the most serious cases, death. Patients typically develop CDI from the use of broad-spectrum antibiotics that disrupt normal gastrointestinal (gut) flora, possibly allowing C. difficile bacteria to flourish. Older patients in particular are at risk for CDI, potentially because of a weakened immune system or the presence of underlying disease. Approximately two-thirds of CDI patients are 65 years of age or older. Historically, approximately 20% to 30% of CDI patients who initially respond to treatment experience a clinical recurrence1.

About Specialised Therapeutics Australia

Specialised Therapeutics Australia (STA), is a bio-pharmaceutical company dedicated to working with leading pharmaceutical companies worldwide to provide acute care therapies for high unmet medical needs to people living in Australia and New Zealand. Our therapeutic portfolio and pipeline encompasses oncology, infectious disease, respiratory, dermatology, endocrinology and central nervous system (CNS). Additional information can be found at www.specialisedtherapeutics.com.au

About Optimer Pharmaceuticals

Optimer Pharmaceuticals, Inc. is a global biopharmaceutical company focused on developing and commercialising innovative hospital specialty products that have a positive impact on society. Optimer developed and commercialised DIFICID® (fidaxomicin) tablets, an FDA-approved antibacterial drug for the treatment of adult patients with Clostridium difficile-associated diarrhoea (CDAD). Optimer has also received marketing authorisation for fidaxomicin tablets in the European Union under the trade name DIFICLIR™. The company is exploring marketing authorisation in other parts of the world where C. difficile has emerged as a serious health problem, including Asia. Additional information can be found at http://www.optimerpharma.com.

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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