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