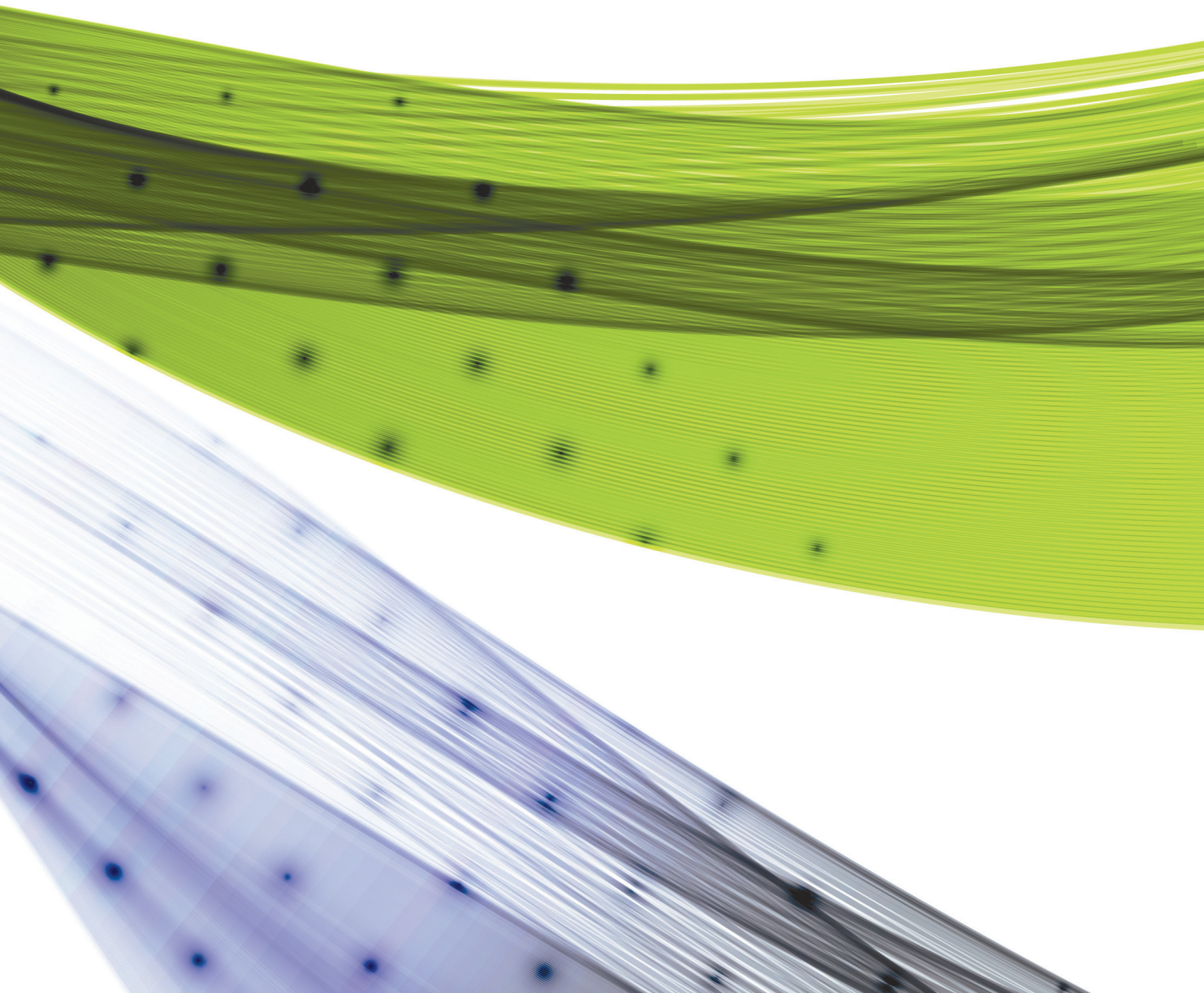
rare cancers AUSTRALIA

# FUNDING FOR TREATMENT OF RARE CANCERS IN AUSTRALIA

A survey of pharmaceutical company employees, physicians and patient/advocacy group members



# FOREWORD

Taxpayer-funded healthcare has always focused on achieving the greatest good for the greatest number of people with the available funds. Nowhere is this more obvious than in the funding of medical research and of medicines. Whilst this has served a portion of the Australian community well, it has inadvertently disadvantaged a large and largely ignored section of the community; those of us who are afflicted with rare or less common diseases particularly cancer.

This survey was commissioned to help understand the impact of the current system on patients with rare or less common cancers. The trigger for Rare Cancers Australia to conduct this survey came during a discussion between oncologists, industry executives and patient groups when a list of drugs was presented that would NOT be made available in Australia because our reimbursement regime made it virtually impossible for these medicines to be approved for funding through the Pharmaceutical Benefits Scheme (PBS).

The results of the survey speak for themselves; patients with rare cancers regularly have to self-fund treatments or go without, the regulatory regime is seen as lacking the flexibility required to deal with rare or less common cancers and there is insufficient funding devoted to research into these diseases.

Whilst the survey presents 3 different perspectives from patients, clinicians and the pharmaceutical industry, there is unanimity in the view that change is needed if we are to treat these Australians as equally as those suffering more common diseases.

This inequity is not a problem that has been caused today or yesterday, it is the result of years of both neglect and of limited options. In the 21st century technology and science have advanced to the point where these patients can have real hope of both improved quality and quantity of life. We now need to change the regulatory framework to embrace this opportunity and care for all Australians equally regardless of their disease.

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# EXECUTIVE SUMMARY

Rare and less common cancers account for approximately 50% of all cancer deaths in Australia[1]. Nevertheless, the proportion of government funding allocated to treating rare cancers is significantly lower than that for common cancers. With less than 20% of cancer research funding and less than 15% of the total PBS cancer spend, many patients are currently undertreated or not treated at all[1].

Rare Cancers Australia (RCA) is a non-profit organization that is committed to improving the lives of rare cancer patients by focusing on early diagnosis and increasing access to improved treatment options. In order to investigate the apparent disparity in treatment and funding between rare and common cancers in Australia, RCA recently conducted a survey amongst pharmaceutical company employees, physicians and patient/advocacy group members.

The results of the survey indicate that access to treatment for rare cancer patients remains a major challenge in Australia. It is extremely difficult to advance rare cancer treatments due to high treatment cost, small patient numbers and challenges associated with clinical trial design. The survey also suggests that Australia has a stringent regulatory environment (with respect to drug approval/registration and securing patient reimbursement), which hinders the development of rare cancer treatments. As a result, pharmaceutical companies are reluctant to apply for drug approval and reimbursement and patients may therefore not have access to specialized or most effective treatments. Together, these have resulted in considerable financial and psychological burdens on rare cancer patients.

Based on the findings from this survey, RCA would like to urge the Australian Government to review the regulatory process for rare cancer drug approval, to increase the availability of PBS-funded treatments and to ensure that patients with rare cancers receive the same level of and access to care and treatment as patients with common cancers.

## Background and Objective

Rare cancers are defined as cancers with an incidence of less than 6 per 100,000 populations per year[2]. Less common cancers are defined as those with an incidence of between 6 and 12 per 100,000 populations[2]. In a typical year in Australia, there are more than 44,000 diagnoses of rare or less common cancers and nearly 24,000 deaths[2]. Together, rare and less common cancers account for 33.8% of the total disease burden in Australia[1].

For Australians with rare and less common cancers, very little help is available, from patient support to new treatment options. Although rare and less common cancers account for the same proportion of cancer deaths in Australia as common cancers[1] funds allocated to these two cancer categories are disproportionate. Less than 20% of cancer research funding and less than 15% of the total PBS cancer spend are allocated for the treatment of rare and less common cancers.[1] In addition, newer treatments are not readily available.

Rare Cancers Australia (RCA) is a non-profit organization that works on behalf of rare cancer patients. RCA is committed to improving the lives of rare cancer patients by focusing on early diagnosis and increasing access to improved treatment options. RCA recently carried out a survey amongst healthcare professionals and patient/advocacy groups to help identify reasons that may be accountable for discrepancies in treatment and funding for rare cancers.

## Survey Methodology

This survey was developed, conducted and analyzed by Comradis® on behalf of RCA. It was distributed via email invitations to pharmaceutical company employees (n=100), physicians (n=50) and patient support/advocacy group members (n=20) in Australia. The survey was open for one month in April-May 2015.

## Results

A total of 127 responses were received (71 full, 54 partial and 2 incomplete responses). Only full responses (n=71) were included in the analysis presented in this report. The full response rates for pharmaceutical company employees, physicians and patient support/advocacy group members were 33%, 42% and 85%, respectively.

## Pharmaceutical Company Employees' Responses

Thirty three employees from AbbVie, Amgen, AstraZeneca, Bayer, Boehringer Ingelheim, GlaxoSmithKline, Janssen, MSD, Merck Serono, Novartis Oncology, Pfizer and Roche responded to this survey. These participants were largely based in Australia, with 2 participants located in UK and USA. The majority of the pharmaceutical employees (60.6%) who participated in this survey hold positions in clinical, communications, corporate affairs, external/government relations, market access, patient/public access and reimbursement. Some of the participants are involved with either commercial (27.3%) or medical (12.1%) roles within the company.

Pharmaceutical company employees agreed that rare cancer patients remain inadequately treated in Australia. The main perceived challenges in advancing rare cancer treatments are high treatment cost and poor clinical data as a result of small patient numbers **(Figure 1)**, which is a key challenge in clinical trial design for rare cancer treatments **(Figure 2)**. Other challenges in clinical trial design include managing crossover trials and the confounding impacts from the crossover. In addition, funding for PBS reimbursement remains the main obstacle for rare cancer patients.

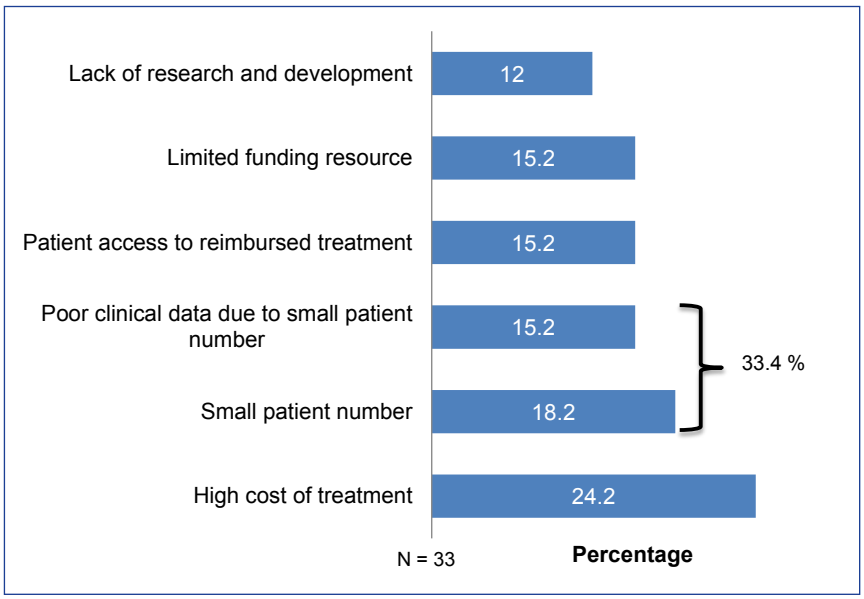



**Figure 1**. Main challenges in advancing rare cancer treatments.

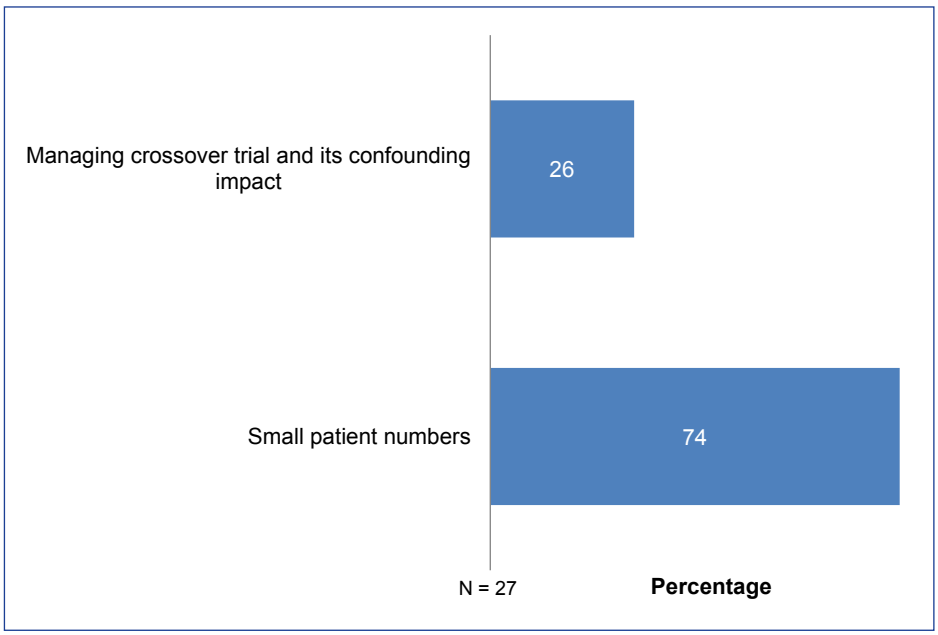



**Figure 2.** Key challenges in clinical trial design for rare cancer treatments.

Participants from pharmaceutical companies also stated that Australia has the most demanding regulatory environment, which is a barrier for rare cancer treatment. Consequently, Australia is ranked in the bottom three (along with the Middle East and Africa) in terms of business priority. Most of the pharmaceutical companies represented in the survey (78.1%) reported that they had decided not to apply for approval/reimbursement for a rare cancer indication because of the anticipated regulatory challenges. The majority of pharmaceutical companies (75.8%) indicated that the standard of evidence required for approval of treatments for common cancer is also applied to rare cancers; however, when compared with other countries, 45.5% of the participants agreed the required standards of evidence for rare cancer treatments are more stringent in Australia. The Australian government still consults the pharmaceutical industry in creating/modifying regulatory policy and approval criteria to some extent.

With respect to patient access programmes, all pharmaceutical company employees agreed that these programmes are important and very useful as they can provide both patients and clinicians with potentially life-saving medicines and new treatment options. However, 20% of employees indicated that these programmes are difficult to sustain in the long run and 23% of them stated that they confer a high risk to the company as obtaining reimbursement is challenging. The participants also added that securing patient reimbursement (36.7%) and high financial risk (33.3%) are the biggest challenges for marketing rare cancer treatments in Australia **(Figure 3)**.

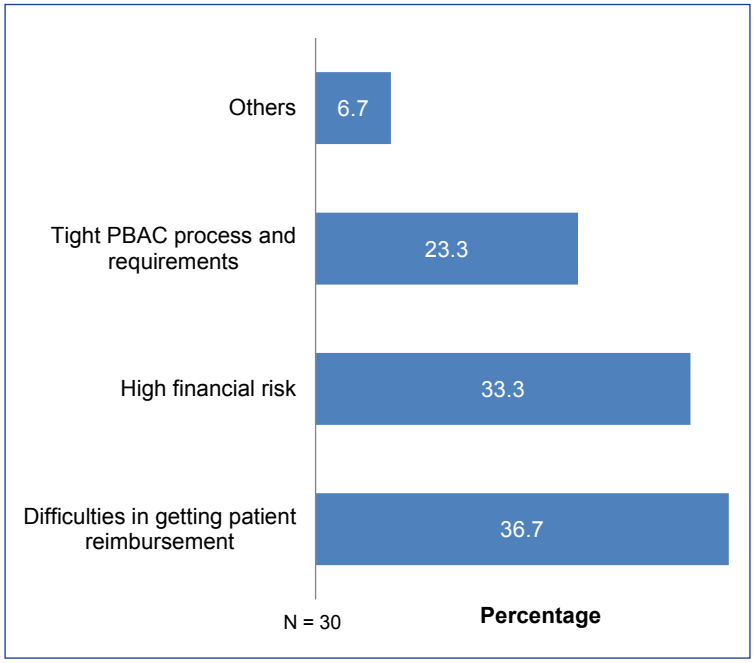



**Figure 3.** Main challenges for marketing rare cancer treatment in Australia. Others include lack of collaboration between investigators, the overall process of drug development and clinical research prioritization. PBAC: Pharmaceutical Benefits Advisory Committee.

"**Reimbursement** system is **not flexible** enough to account for the unique challenges of rare cancers"

"New cancer treatments are expensive and the government is faced with unmet patient needs. It is difficult for everyone"

"Japan and China are becoming higher priority countries within company's global strategy"

"**Australia** has become the **most challenging market** outside of Korea and New Zealand to obtain reimbursement"

"While Medicines Australia (MA) is often consulted, MA has limited engagement with membership, aside from board members and a few industry members who have held positions in committees for many years and have **little motivation for real change** (just a lot of talk)"

"Accruing patient numbers takes time – increased expense quite often lack the consensus clinical guidelines, thus creating challenges in interpreting information across different studies"

"The issue is whether this 'standard of evidence' is appropriate, especially in terms of cost-effectiveness assessments. These rely heavily on demonstrating overall survival, which often cannot be ethically demonstrated using randomized control design studies"

"The biggest challenge is that we can only rely on clinical studies to support reimbursement and submission; with **rare cancers**, this is **extremely difficult** (eg no comparator arm, open label design, etc)"

"Patient access programmes can be great for patients if they provide cost effective access. However, cost effective is different for every person's circumstances. They can also become very expensive for pharmaceutical companies"

**Pharmaceutical company employees**

## Physicians' Responses

Twenty Australian physicians responded to the survey. The majority (70%) of these physicians are from university and research hospitals, whereas 55% and 45% of them are from public hospitals and private clinics, respectively. More than half of the surveyed physicians (55%) treat digestive organ cancers (excluding colon and rectum), and nearly half of them (45%) treat cancers of mesothelial, connective/soft tissue, female reproductive organs and ill-defined, secondary/unspecified types of cancers. A small proportion of these physicians also treat lip/oral cavity/pharynx (10%), skin (15%), eye/brain/other parts of nervous system (15%) and thyroid/other endocrine glands (15%) cancers in their clinics.

From a physician's perspective, the top 3 reasons for poor access to medications for rare cancers in Australia are:

1. inadequate research efforts for new treatments
2. insufficient funding by the PBS
3. lack of consultation with the rare cancer community.

The physicians indicated that both the PBS and Therapeutic Goods Administration (TGA) have strict regulatory environment that creates a hurdle for rare cancer treatment, resulting in the unavailability of some rare cancer medications in Australia. The physicians also believed that the structure, processes and/or focus of the TGA and PBS have negatively affected treatment access for rare cancer patients. Nearly half of the physicians agreed that 50% of their rare cancer patients have treatments that are not funded by the PBS for the patients' disease indication. Furthermore, the majority of physicians (71.4%) agreed that they should be given the option to apply for TGA approval for an off-label indication in rare cancers.

Physicians also believed that patient-focused assisted programmes are necessary to improve treatment access. More than half of the physicians (66.7%) who participated in the survey agreed that treatment access for rare cancer patients is poorer than that for common cancer. Although funding continues to be an issue for patients, 60% of physicians have helped only <50% of their patients apply for alternative funding. When asked about their funding resources, 45% of the physicians said that they often apply directly to pharmaceutical company on compassionate grounds, whereas 68.4% of them have never applied to the Australian's Life Saving Drugs Program **(Figure 4)**. Participating physicians were also actively looking for treatment access and options for their patients. These physicians considered consultations with other physicians and internet-based continuing medical education as the most accessible resources in getting information about rare cancer treatment options **(Figure 5)**.

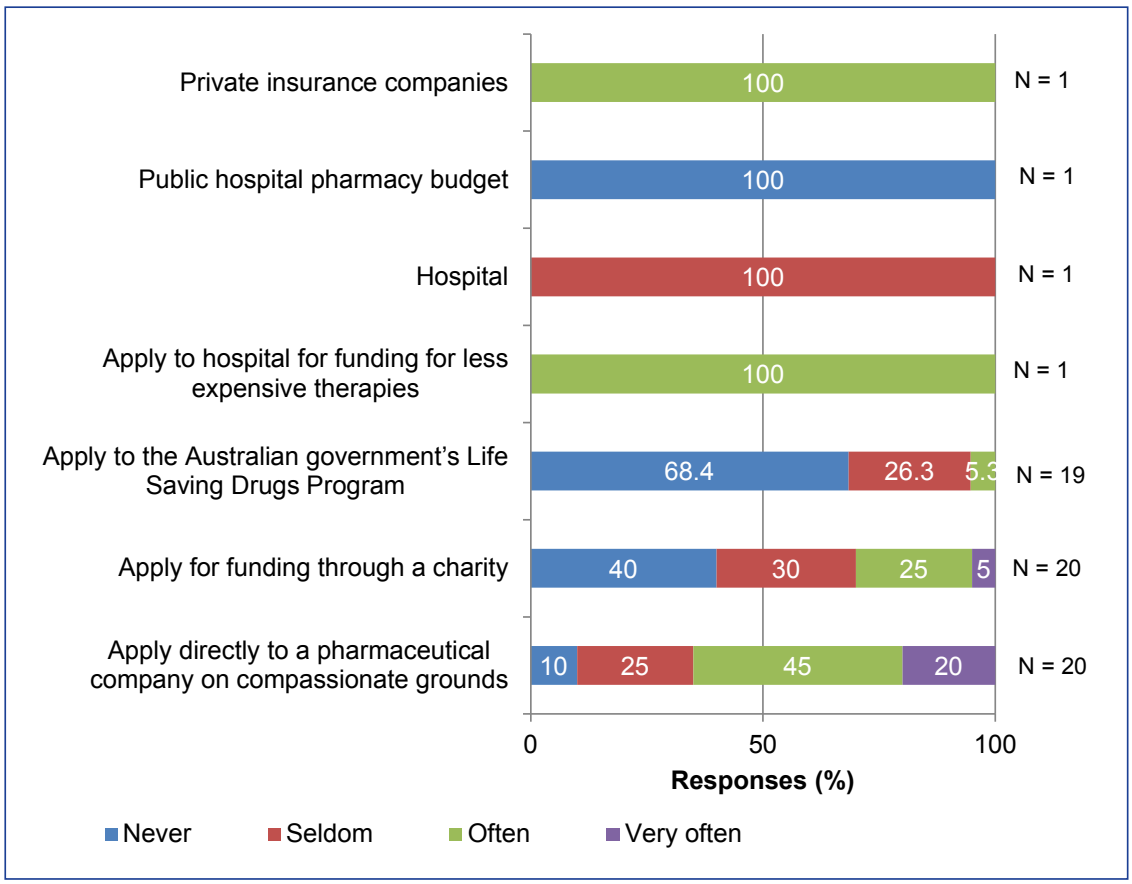



**Figure 4.** Frequency of funding application through different resources.

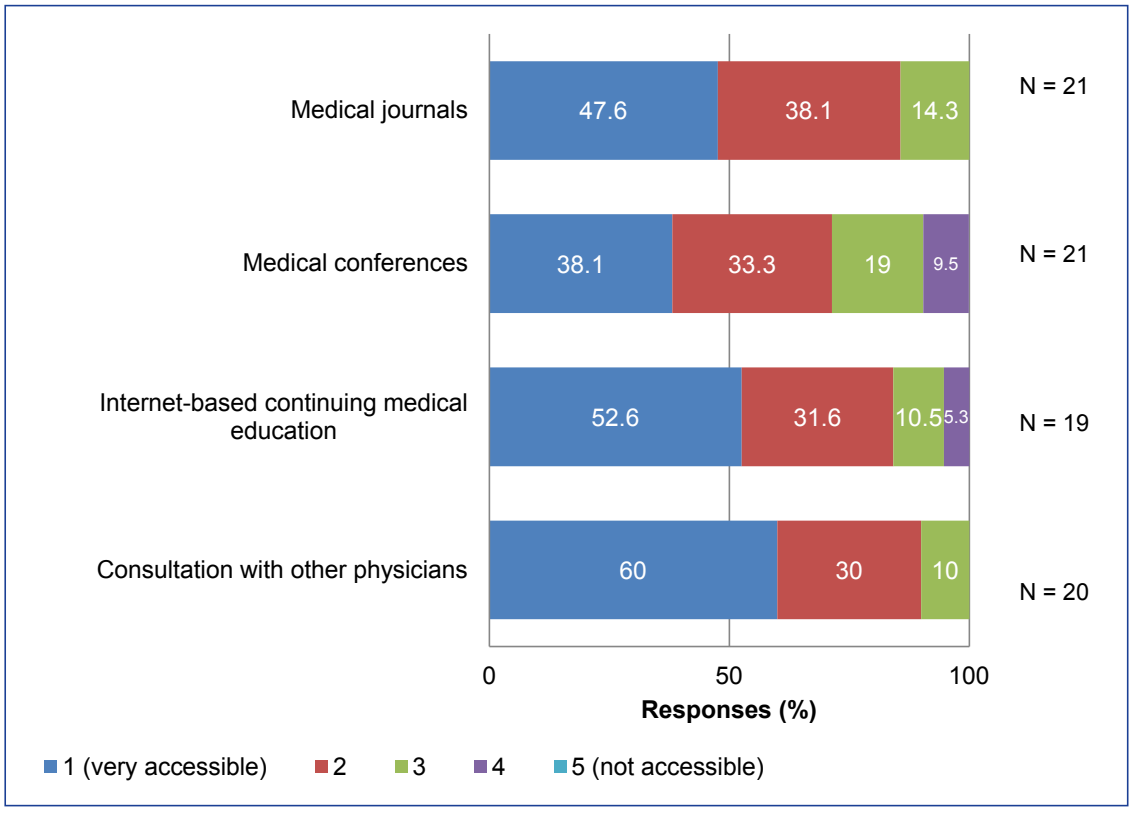
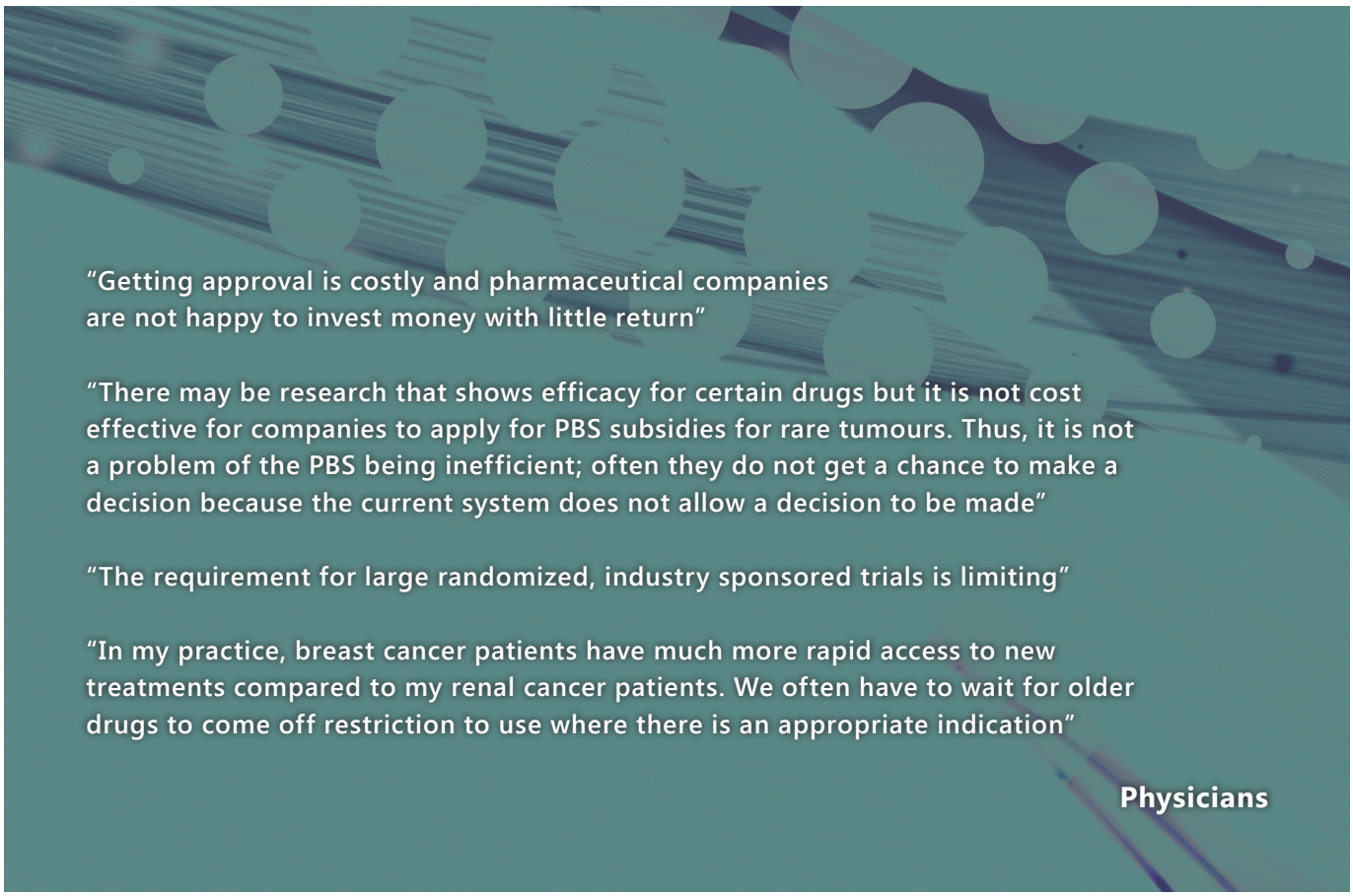



**Figure 5.** Accessibility on information and resources about rare cancer treatment options.




## Patient Support/Advocacy Groups' Responses

Seventeen complete responses were received from Asbestosis and Mesothelioma Association of Australia, Brain Tumour Alliance Australia, CanSpeak, Cancer Drug Alliance, Cancer Voices, John Logan Cancer Treatment Foundation, Leukaemia Foundation, Lung Foundation Australia, Myeloma Australia, Ovarian Cancer Australia, Rare Cancers Australia and The Unicorn Foundation, all based in Australia. Most of these organizations focus on patient advocacy/government lobbying (88.2%) and raising public awareness about rare cancers (58.8%). Some of these groups also focus on fundraising for rare cancer treatment (35.3%) and research (47.1%). Nearly half (47%) of these organizations represent both rare and common cancers. Approximately 30% of these organizations represent cancers of digestive organs (excluding colon and rectum) and respiratory/intrathoracic organs. A small number of these organizations represent cancers of the lip/oral cavity/pharynx (12%), bone/cartilage (12%) and urinary tract (12%).

According to the patient support/advocacy groups, the top 3 reasons for poor access to medications for rare cancers in Australia are:

1. lack of research into new treatments for rare cancers
2. insufficient funding by the PBS
3. slow approval process by the TGA.

Similar to pharmaceutical company employees and physicians, patient/advocacy group members also stated that poor access to treatment for rare cancer patients remains a significant problem. Unfortunately, the majority (64%) of patient/advocacy organizations do not have financial resources to assist with cost of treatment for rare cancer patients. These groups considered increased disease awareness and communications between government and pharmaceutical industries, as well as lobbying effort from the physicians as the most effective ways to improve treatment access for rare cancer patients.

Rare cancers have a significant impact on patients' quality of life. The majority (94%) of patient/advocacy groups are aware of rare cancer patients' inability to access prescribed medications due to financial constraints. When funding and/or treatment are not available from any source, 50% of patients choose to continue with PBS-listed medications or seek compassionate access **(Figure 6)**. The majority (80%) of rare cancer patients must forgo non-necessities and borrow money from friends or family to receive treatment **(Figure 7)**.

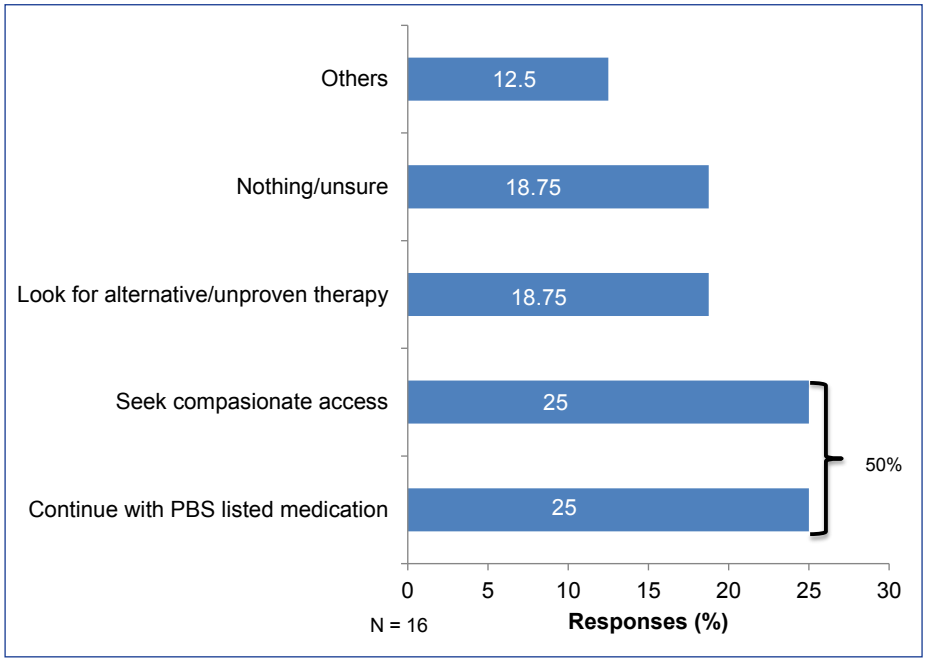



**Figure 6.** Different types of actions taken by rare cancer patients when funding/treatment is not available from any source.

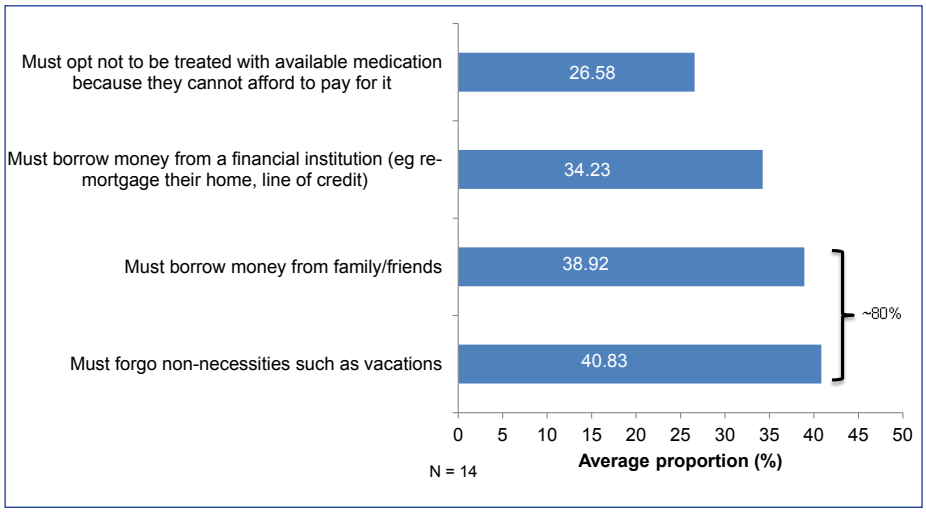



**Figure 7.** Types of financial challenges encountered by rare cancer patients when undergoing treatment for the disease.

Over 50% of rare cancer patients are unable to work (lost their job) and experience depression during the course of their disease **(Figure 8)**. In addition, the proportions of patients who encounter mental and personal challenges are greater for rare cancers than common cancers.

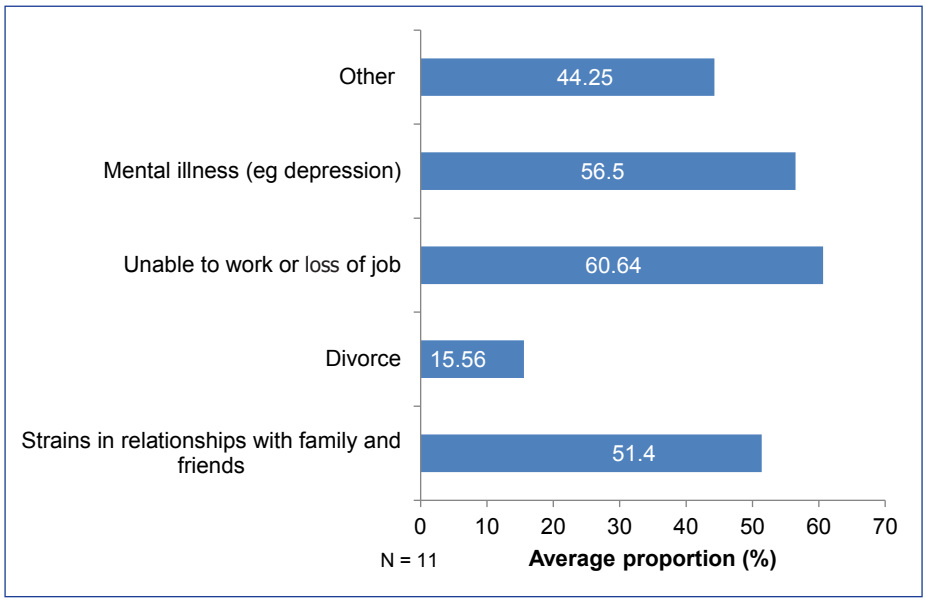



**Figure 8.** Proportions of are cancer patients who encounter mental/personal challenges during the course of their disease.

Patient advocacy groups believed that there is a need for education for patients about the PBS scheme as most patients (70.6%) do not understand the process of listing medications on the PBS. In addition, they believed that better education and public awareness programme may improve patients' understanding of the PBS listing process **(Figure 9)**.

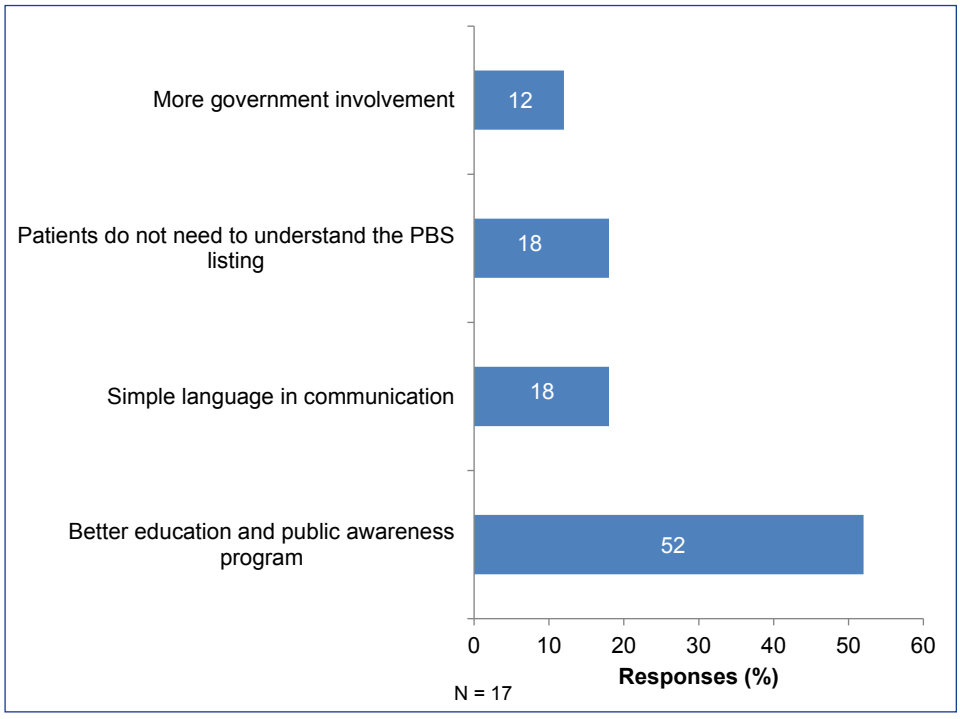



**Figure 9.** Ways to help improve patients' understanding of the PBS listing process.

"RCA works to help both today's and tomorrow's patients through advocacy and fundraising"

"We are receiving increasing numbers of calls and emails relating to financial distress, patients needing to make large copayments even under compassionate access schemes and these include requests for financial assistance"

"Rare cancers don't have the evidence due to lower number or poorer outcomes/survivors"

"There are a number of experimental therapies being conducted overseas, which are not available in Australia"

"The PBS process is too slow – this has been identified on many occasions"

"Negotiation between pharmaceutical and PBS is often tied up for months, delaying access to patients"

"Most rare cancers have small population groups or markets making it unattractive for companies to apply for PBS listing and if they do, it is difficult for them to prove cost-effectiveness"

"Some patients will look overseas to find a clinical trial or experimental treatment. Generally patients will try and do whatever they can to have more time with their families"

"Cure is not always the priority; quality of life and more time are often the most important factors"

"Rare cancer patients can feel isolated, less supported and understood due to the rarity of their condition"

"There is a need for a more transparent approach. The current PBS system is opaque and unnecessarily complex using highly technical terminology that an average person would not understand"

**Patient support/advocacy group members**

## Challenges for Rare Cancer Patients: The Overall Perspective

### PHARMACEUTICAL COMPANY EMPLOYEES

**Almost 90%** of pharmaceutical company employees agree that **lack of funding for PBS** reimbursement is the main **barrier** for patient's access to treatment **(Figure 10)**.

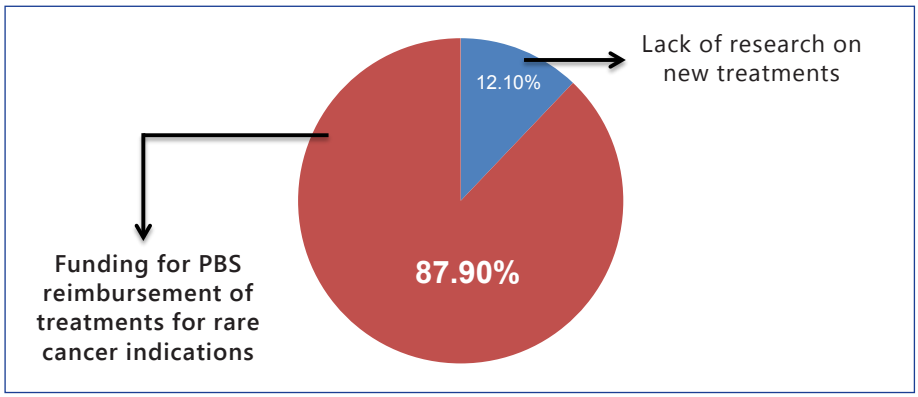



**Figure 10.** Obstacles to treatment access for rare cancer patients

### PHYSICIANS

**Nine out of ten** physicians stated that the **stringent and complex** regulatory environment in Australia has a **negative impact** on rare cancer patients **(Figure 11)**.

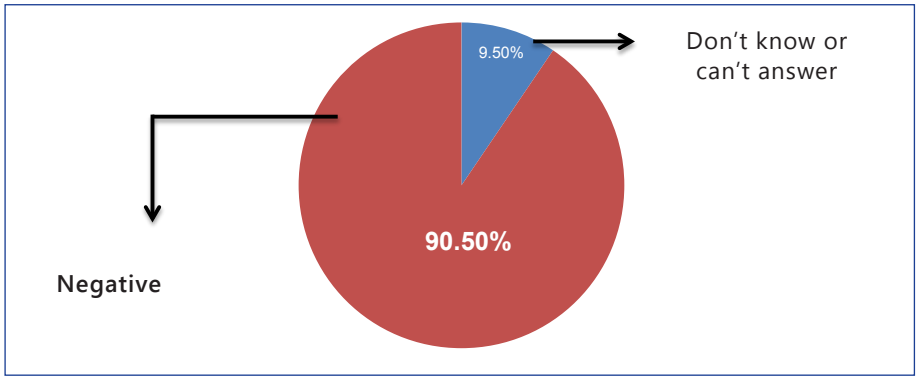



**Figure 11.** The impact of structure, processes and/ or focus of the TGA and PBS on rare cancer patients.

### PATIENT /ADVOCACY GROUP MEMBERS

**More than 80%** of patient/advocacy group representatives believed that patients with **rare cancers** have **poorer** access to treatment compared with those with common cancers **(Figure 12)**.

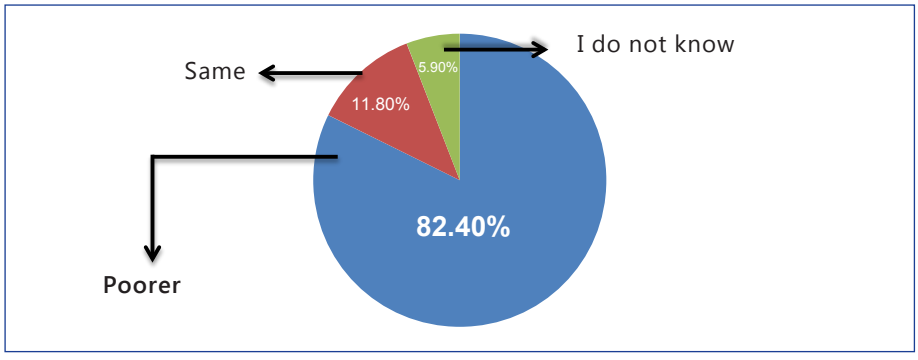



**Figure 12.** Comparison of access to treatment for patients with rare cancers versus those with common cancers.

## Conclusion

Pharmaceutical company employees, physicians and patient advocacy groups agreed that treatment access for rare cancer patients remains a problem in Australia. It is extremely difficult to advance rare cancer treatments due to high treatment cost, small patient numbers, and challenges associated with clinical trial design and poor clinical data. Both physicians and patient/advocacy groups agreed that inadequate research efforts for new treatment and insufficient funding by the PBS are the main reasons for difficulty in accessing medication for rare cancer treatments in Australia.

The strict regulatory environment set forth by the Australian PBS and TGA is a barrier for making rare cancer treatments available. Such demanding regulations have resulted in:

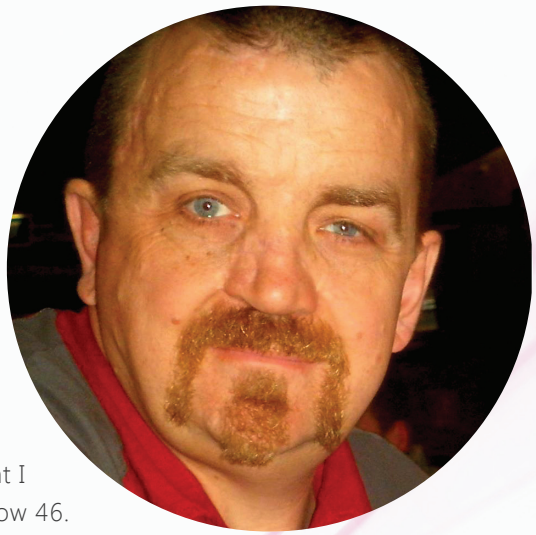
- the unavailability of certain medications for rare cancer patients;
- pharmaceutical companies' hesitation in applying for approval and reimbursement
- financial constraints for rare cancer patients accessing treatments not listed by the PBS.

As funding for treatment continues to be a challenge, this has placed a financial burden on patients and affected their quality of life. Rare cancer patients often choose to continue with PBS-listed medications, despite the fact that these drugs may not be the most effective treatment options. As some patients may become unable to work and will need to seek compassionate access, their mental health is also affected by the disease. Many of these patients have experienced depression during the course of their illness.

Based on the results of this survey, RCA recommends that it is time for the Australian Government to:

- assist with research to identify novel treatment for rare cancers
- relax the regulatory environment for rare cancer drug approval and registration
- increase the availability of PBS funded treatment
- provide equal levels and access to care and treatment for all types of cancer.

Rare and less common cancers present great challenges to the health system. RCA hopes that the above steps can have positive impacts in improving outcomes for rare cancer patients in Australia.



## Geoff

I am 46 and I have Gorlins Syndrome. I was born with a number of physical abnormalities. I had an extra half toe on my left foot and an extra part formed finger on my right hand. Both these were removed soon after birth.

My left leg from the shin down was severely turned, facing the right heel. It took over 13 operations to correct. I endured toe to hip plaster for several months. Needless to say, I came into this world facing great hardships. It was a difficult time for my family. With endless surgery related problems, doctors stated that I would be lucky to reach 21. Fortunately they were wrong, I am now 46.

To date, I have had over 176 surgeries. I have since stopped counting. A good year is one that sees me having 3 surgeries, a bad year has been one that includes 11 operations.

Cysts are an ongoing issue with my disease. I have lost count on the numerous teeth and jaw cysts that have been removed. These procedures have resulted in an uneven bite and cracked teeth. My left knee has seen a total of 4 cysts removed. Scars are a permanent mark on my body. They are a reminder of the physical and emotional pain I have endured in my battle to fight this disease. Growing up was extremely hard. Time off school and the constant bullying was an extra unnecessary burden.

The real burden at the time was the little information and support available to someone like me. There were many questions and very few answers. But I had to live my life and make the most of it. Through IVF and a donor sperm, my wife and I were able to conceive. My son is now 14 years old. At such a young age, he has witnessed the devastating trauma that cancer brings.

I need treatment and I know I will improve with the right drugs but I simply cannot afford the cost.

## Louisa

My name is Louisa and I am a 35 year old mother of two absolutely gorgeous children. On 23 June 2014, just two days before my son's 13th birthday, I was given the devastating news that the extreme pain I was feeling in my shoulder was not caused by something muscular but by Osteosarcoma, a rare form of cancer.

Not long after this devastating diagnosis, I commenced ten months of chemotherapy treatment. So far I have been given 5 different kinds of chemo, some have helped to slow the progress of my cancer, but have unfortunately done a lot of damage to the rest of my body and were therefore not sustainable. Others did nothing to stop my cancer.

Recently I have been given the chance to try a new treatment called Keytruda. There have had some real successes with this treatment for other patients with advance melanoma and my oncologist believes it might work for sarcomas too. This is a drug trial of one and hopefully if I get some success with it, others with sarcoma may get the chance to trial it too.

The difficult thing is that until we can get it onto the PBS, this treatment will cost approximately $6000 a round. It's so heartbreaking to be given the hope of a treatment that could change your life expectancy but then wonder how on earth you will afford it.

I'm really hoping that this new treatment will extend my life. My greatest wish is for more time to watch my precious children grow up and more time to grow old with my husband, the way I had anticipated on our wedding day. I have so much to live for and I so desperately want to live.

## Nick

I was diagnosed with Renal Clear Cell CarcinBoma on 8th March 2013 and was admitted to hospital almost immediately for a nephrectomy on my left kidney. There were two tumours 85 x 70 x 60mm in the upper pole and 40 x 25 x 15 mm in the lower pole. Both were Fuhrman grade 2 and they suspected renal sinus invasion in the upper pole block 7 and a loose tumour "plug" in the lumen of the relatively large intra-renal vein.

All this information was like a foreign language to me. I had only made an appointment with the doctor because of an odd feeling in my scrotum and he had sent me to have an ultrasound. Cancer was not the diagnosis I was expecting and it was like being hit between the eyes with a sledge hammer and it continues to be this way every single day 20 months later.

After the removal of my kidney in March 2013, I returned home and made the scheduled appointment with my urologist to find out the results. The urologist was hopeful that it was Stage II Renal Cell Carcinoma and was happy that all the tumours had been removed successfully, but just to be certain referred me to an oncologist to get a further CT scan on my lungs. We went home and celebrated a lucky escape.

However, the scans indicated that the cancer had already metastasized to my lungs and there were multiple tumours of varying sizes present. I commenced oral chemotherapy using a drug called Zotrient which immediately turned my hair white, gave me mouth ulcers and nearly killed me with transient hepatitis. All the time the tumours were growing and multiplying every day.

In September 2013, I commenced a new drug Sutent and it too presented its own set of side effects but fortunately this time my liver remained unaffected so I was able to continue the treatment. This treatment worked well and every CT scan presented shrinkage of tumours and by late January 2014, only 3 lung tumours remained and they were quite small.

But in June I started to develop headaches and I was losing the ability to hold or grip. A CT scan of the brain showed the cancer had now metastasized to my brain and there were in fact 2 tumours. I was admitted to hospital immediately for brain surgery. The operation was a success but not without an agonising recovery process.

In the meantime further scans revealed that the lung tumours were multiplying quickly and increasing in size due to not receiving any chemo while recovering from surgery and commencing whole brain radiotherapy (WBR). It was decided to immediately commence chemo using Afinator in conjunction with WBR even though this isn't usually recommended. The next CT scan in September 2014 once again showed wide spread disease in the lungs and 4 new tumours in the brain.

Luckily for me in the week preceding my admission to Palliative Care my oncologist had given me the option to no longer receive treatment or give it one last shot with a drug that isn't on the PBS. Although it is a very expensive drug it was my only option and after much debate with myself I finally agreed to commence treatment.

This drug should be made readily available to not just me but everyone that has RCC, it is cruel to have a drug that can potentially give quality and longevity of life to RCC sufferers but then attach a price tag that makes it impossible to obtain.

Nick Collings sadly passed away on Saturday 24 January 2015.

## Lillian

My world shattered just days after my 34th birthday. I was diagnosed with advanced lung cancer. I am not about to let this diagnosis define my whole life. I aim to live with cancer and with love and thankfulness in my heart. You see, I have so much to be thankful for. God has given me a loving husband who is my rock and best friend, an amazing 3-year-old daughter who is our everything, a wonderful extended family that I could not have picked better, beautiful friends and colleagues and a dream job as a social justice lawyer that I am deeply passionate about.

It was easy to miss the signs. I had had a cough for two months prior to my diagnosis, but had dismissed it as a post-viral cough. Any discomfort I had I also took as first trimester morning sickness. We had just started to announce to our family and friends the wonderful news that we were expecting our very much longed for second child. This pregnancy compounded on our situation as we soon faced not only the aftermath of the cancer diagnosis and what that meant, but also an incredibly agonising decision regarding the pregnancy, a decision no parent should ever have to make. We struggled with finding a path forward as doctors advised us that waiting until after pregnancy for treatment was not an option and that terminating the pregnancy would be the best course of action.

As the days went by, further testing showed I had developed a rare form of lung cancer caused by the mutation of the ROS1 gene, a mutation that only 1-2% of non-small cell lung cancer patients have, and one often found in young non-smokers like myself. The target therapy drug Crizotinib (Xalkori) has been found in recent research to be an effective antitumor medicine for this type of mutation and also has minimal side effects compared to other types of chemotherapy.

In a situation where the prognosis is generally dismal, measured in months for advanced stage cancer, Crizotinib has shown potential to extend the prognosis to years. Unfortunately, this medicine is not on the PBS and as there are no current trials available for my mutation type. It will cost us approximately $7,400 a month to access the medication that I need.

I look to this medicine as my hope. I hope to be there to send my daughter off on her first day of school with tears in my eyes, I hope to celebrate many more birthdays with the people I love, and most of all, I hope to share many more years of tears and laughter with my husband, the love of my life, as we navigate this journey together.

# ACKNOWLEDGMENTS

This report has been prepared by Rare Cancers Australia Ltd (RCA) to provide a better understanding of the challenges facing Australians with a rare or less common cancer today.

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- Comradis

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## References


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## Appendix A – Survey Questions

| Pharmaceutical company employees | |
|---|---|
| 1. What is your primary role? | ❑ Commercial<br>❑ Medical<br>❑ Other (please specify) _____ |
| 2. What company do you work for? | [open answer] |
| 3. Are you based in Australia? | ❑ Yes<br>❑ No (where?)_____ |
| 4. What are the main challenges in advancing rare cancer treatments? | [open answer] |
| 5. From your point of view, what is the main obstacle to treatment access for patients with rare cancers in Australia? (please choose one) | ❑ Regulatory approval of new treatments<br>❑ Lack of research on new treatments<br>❑ Funding for PBS reimbursement of treatments for rare cancer indications<br>❑ Other (please specify) _____ |
| 6. Please rank the following markets in order of business priority from your company's global perspective.<br><br>Drag items from the left-hand list into the right-hand list to order them. | ❑ Africa<br>❑ Asia<br>❑ North America<br>❑ Australia<br>❑ South America<br>❑ Middle East<br>❑ Europe |

| | |
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| 7. Please rank the following regions in order of most challenging to least challenging regulatory environment.<br><br>Drag items from the left-hand list into the right-hand list to order them. | ❑ Africa<br>❑ Asia<br>❑ North America<br>❑ Australia<br>❑ South America<br>❑ Middle East<br>❑ Europe<br>❑ Other (please specify) _____ |
| 8. To what extent does the Australian government consult the pharmaceutical industry about creating or modifying regulatory policy, approval criteria, etc? | ❑ Extensively<br>❑ To some extent<br>❑ No consultation<br>❑ Don't know |
| 9. What is/are the key challenge(s) in clinical trial design for rare cancer treatments? | [open answer] |
| 10. Is the standard of evidence required for approval of common cancer treatment in Australia also applied to rare cancers? | ❑ Yes<br>❑ No<br>❑ Don't know |
| 11. Is the standard of evidence required in Australia for rare cancer treatment different from that in other countries? | ❑ Yes, requirements are more stringent in Australia<br>❑ Yes, requirements are less stringent in Australia<br>❑ About the same<br>❑ Don't know |
| 12. Do companies ever decide not to apply for approval or reimbursement for a rare cancer indication in Australia because of anticipated regulatory difficulties? | ❑ Yes<br>❑ No<br>❑ Don't know<br>❑ Comments: |
| 13. In your opinion, what is the single biggest challenge in getting rare cancer treatments to market in Australia? | [open answer] |
| 14. What is your view of the role of patient access programmes? | [open answer] |

| Physicians | |
|---|---|
| 1. Please describe your practice (please check all that apply). | ❑ Private clinic<br>❑ Public clinic<br>❑ Rural practice<br>❑ University/research hospital<br>❑ Urban practice<br>❑ Public hospital<br>❑ Other |
| 2. What types of rare cancers do you encounter in your practice? (please check all that apply) | ❑ All cancer types (rare and common)<br>❑ Common cancers only<br>❑ Lip, oral cavity and pharynx<br>❑ Digestive organs (excluding colon and rectum)<br>❑ Respiratory and intrathoracic organs (excluding lung and trachea)<br>❑ Bone and articular cartilage<br>❑ Skin (excluding melanoma)<br>❑ Mesothelial, connective, and other soft tissue<br>❑ Female genital organs<br>❑ Male genital organs excluding prostate<br>❑ Urinary tract<br>❑ Eye, brain and other parts of the central nervous system<br>❑ Thyroid and other endocrine glands<br>❑ Ill-defined, secondary and unspecified sites<br>❑ Haematological malignancies (excluding Non-Hodgkin's lymphoma)<br>❑ Independent (primary) multiple sites |
| 3. How does treatment access compare between patients with rare cancers and those with common cancers? | ❑ Poorer for patients with rare cancers<br>❑ Same<br>❑ Better for patients with rare cancers<br>❑ I do not know |
| 4. Please rank in order the top 3 reasons for rare cancer patients' difficulty accessing medication<br><br>Drag items from the left-hand list into the right-hand list to order them. | ❑ Lack of research into new treatments for rare cancers<br>❑ Slow approvals process by the Therapeutic Goods Administration (TGA)<br>❑ Lack of funding by the PBS<br>❑ Inefficient approvals process by the PBS<br>❑ Lack of consultation with the rare cancer community<br>❑ Other; please specify_____ |

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| 5. How do you obtain information and resources about rare cancer treatment options?<br><br>Please rate the following in terms of accessibility on a scale of 1 to 5 (1=very accessible; 5=not accessible) | ❑ Consultations with other physicians<br>❑ Internet-based continuing medical education<br>❑ Medical conferences<br>❑ Medical journals<br>❑ Other (enter another option) |
| 6. How much impact does the regulatory environment have on the unavailability of certain medications for rare cancers in Australia? | ❑ Yes, very much<br>❑ Yes, somewhat<br>❑ No, very little<br>❑ Not at all<br>❑ I do not know |
| 7. How are patients with rare cancers affected by the structure, process and/or focus of the TGA and PBS? | ❑ Positively<br>❑ Negatively<br>❑ No effect<br>❑ Don't know or can't answer |
| 8. For what proportion of your rare cancer patients are there treatments available but not funded by the PBS for the patient's disease indication? | ❑ ____% |
| 9. For what proportion of your rare cancer patients do you help apply for alternative sources of funding? | ❑ ____% |
| 10. How often do you apply to each of the following sources for funding?<br><br>Apply directly to a pharmaceutical company on compassionate grounds<br><br>Apply for funding through a charity<br><br>Apply for the Australian government's Life Saving Drugs Program<br><br>Other (enter another option) | ❑ Options:<br>❑ Never<br>❑ Seldom<br>❑ Often<br>❑ Very often |
| 11. For what proportion of your rare cancer patients do you prescribe off-label treatment? | ❑ ____% |
| 12. Should oncologists be given the option to apply for TGA approval for an off-label indication in rare cancers? | ❑ Yes<br>❑ No<br>❑ I do not know |

| Patient/advocacy group members | |
|---|---|
| 1. What is your organization's name? (for survey tracking only; will not be published without permission) | ❑ [open answer] |
| 2. What is your organization's focus? (please check all that apply) | ❑ Public awareness about rare cancers<br>❑ Fundraising for rare cancer treatment<br>❑ Fundraising for rare cancer research<br>❑ Patient advocacy (government lobbying)<br>❑ Other (please specify):_____ |
| 3. What types of cancers does your organization represent? (please check all that apply) | ❑ All cancer types (rare and common)<br>❑ Common cancers only<br>❑ Lip, oral cavity and pharynx<br>❑ Digestive organs (excluding colon and rectum)<br>❑ Respiratory and intrathoracic organs (excluding lung and trachea)<br>❑ Bone and articular cartilage<br>❑ Skin (excluding melanoma)<br>❑ Mesothelial, connective, and other soft tissue<br>❑ Female genital organs<br>❑ Male genital organs excluding prostate<br>❑ Urinary tract<br>❑ Eye, brain and other parts of the central nervous system<br>❑ Thyroid and other endocrine glands<br>❑ Ill-defined, secondary and unspecified sites<br>❑ Haematological malignancies (excluding Non-Hodgkin's lymphoma)<br>❑ Independent (primary) multiple sites |
| 4. Are you aware of rare cancer patients who have not been able to access prescribed medications because of financial constraints? | ❑ Yes<br>❑ No |
| 5. How does treatment access compare between patients with rare cancers and those with common cancers? | ❑ Poorer for patients with rare cancers<br>❑ Same<br>❑ Better for patients with rare cancers<br>❑ I do not know |
| 6. Please rank in order the top 3 reasons for rare cancer patients' difficulty accessing medication.<br><br>Drag items from the left-hand list into the right-hand list to order them. | ❑ Lack of research into new treatments for rare cancers<br>❑ Slow approvals process by the TGA<br>❑ Lack of funding by the PBS<br>❑ Inefficient approvals process by the PBS<br>❑ Lack of consultation with the rare cancer community<br>❑ Other; please specify:_____ |

| | |
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| 7. What proportion of your organization's budget is spent on funding treatments for rare cancer patients? | ❑ ____% |
| 8. What proportion (%) of your rare cancer patients encounters the following financial challenges when undergoing treatment for a rare cancer? | ❑ ____% must forgo non-necessities such as a vacation<br>❑ ____% must borrow money from family/friends<br>❑ ____% must borrow money from a financial institution (eg re-mortgage their home, line of credit)<br>❑ ____% must opt not to be treated with available medication because they cannot afford to pay for it |
| 9. What do patients do when funding for their medication is not available from any source? | ❑ [open answer] |
| 10. What proportion (%) of your rare cancer patients encounters each of the following significant personal challenges during the course of their disease? | ❑ ____% Strains in relationships with family and friends<br>❑ ____% Divorce<br>❑ ____% Unable to work or lost a job<br>❑ ____% Mental illness (eg depression)<br>❑ Other; please specify:_____ |
| Are these proportions greater than for common cancer patients? | ❑ Yes, much greater<br>❑ Yes, somewhat greater<br>❑ About the same<br>❑ No, somewhat less<br>❑ No, much less |
| 11. Do patients understand the process of listing medications on the PBS? | ❑ Yes, very well<br>❑ Yes, somewhat<br>❑ No, very little<br>❑ No, not at all |
| 12. How could patients' understanding of the PBS listing process be improved? | ❑ [open answer] |
| 13. Which of the following measures would be effective to increase treatment availability for rare cancer patients?<br><br>Please rate each one on a scale of 1 to 5 (1=very effective; 5=not effective) | ❑ Increased public awareness of rare cancers<br>❑ Government lobbying by physicians' groups to increase speed of PBS listing<br>❑ Increased government consultation with pharmaceutical companies to improve research and access<br>❑ Other, please specify:_____ |

# ABOUT RCA

Rare Cancers Australia Ltd (RCA) is a charity whose purpose is to improve awareness, support and treatment of Australians with rare and less common (RLC) cancers. Every year there are over 44,000 diagnoses of RLC cancers and around 24,000 deaths.

As distinct from common cancers (breast, prostate, bowel, lung and melanoma) there is very little patient support offered to RLC cancer patients. RCA works tirelessly to ensure that these cancers that impact so many lives will never be forgotten or ignored again.

Rare Cancers Australia Ltd is governed by a Board of Directors and is classified as a Health Promotion Charity by the Federal Government. All contributions are fully tax deductible. For more information, please visit www.rarecancers.org.au

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