rare cancers AUSTRALIA

RARE SOLUTIONS

A TIME TO ACT

PROGRESS UPDATE

FOREWORD

In August 2017, Rare Cancers Australia presented our Rare Solutions Report to the Federal Government in the hope that it would engender a sustained and meaningful response for patients, and directly result in better care and treatment. It is with great satisfaction that we can report remarkable progress in many areas, although in others there is still work to be done.

Research

During 2017 and 2018 we have seen a number of forward funding commitments for rare cancers and rare diseases, totalling $248 Million over 5 years. Additionally, the Australian Genomics Cancer Medicine Program has been fully funded with $50 Million over 5 years. These are very welcome initiatives and will result in clinical trial participation becoming available to thousands of rare cancer patients. There is still progress to be made in equalising the proportion of NH&MRC funding for rare cancer research with burden of disease but we are significantly advanced over previous years.

Please meet Matthew. *Matt is a young sarcoma patient whose family have paid in excess of $500,000 to treat him in Australia and overseas. At the last minute, he was accepted onto a trial in Australia and has responded extremely well. He is now back working as an engineer and relishing the ability to participate in his community. Clinical trials provide not only evidence for health professionals and Government but give genuine hope and opportunity to Matt and other rare cancer patients.*

Registration and Reimbursement of Cancer Medicines

The past 12 months also saw the introduction of priority and provisional registration processes by the Therapeutic Goods Administration (TGA) allowing expedited introduction of proven cancer treatments into Australia. This is a great initiative but now needs to be mirrored with similar processes relating to PBS re-imbursement. A significant step forward was Minister Hunt's call on the Pharmaceutical Benefits Advisory Committee (PBAC) to conduct an inquiry into streamlined 'multi-tumour subsidy' options for PD-1 and PD-L1 checkpoint inhibitors. This initiative, if enacted, will be a significant step forward in the availability of treatment for rare cancer patients. However, it is not the full story. There are many targeted medicines that have efficacy across multiple tumour types and we look to a more complete streamlined subsidy process being introduced.

Please meet Carol. *An elderly lady who is in danger of selling her house and has exhausted her life savings paying for a PD-1 checkpoint inhibitor treatment. She has a salivary gland cancer and has responded brilliantly to treatment. The treatment is available on the PBS for other cancer types, but not for 'hers'. The absence of any compassion being shown by the pharmaceutical company or anyone else is a chilling reminder of the need for this change.*

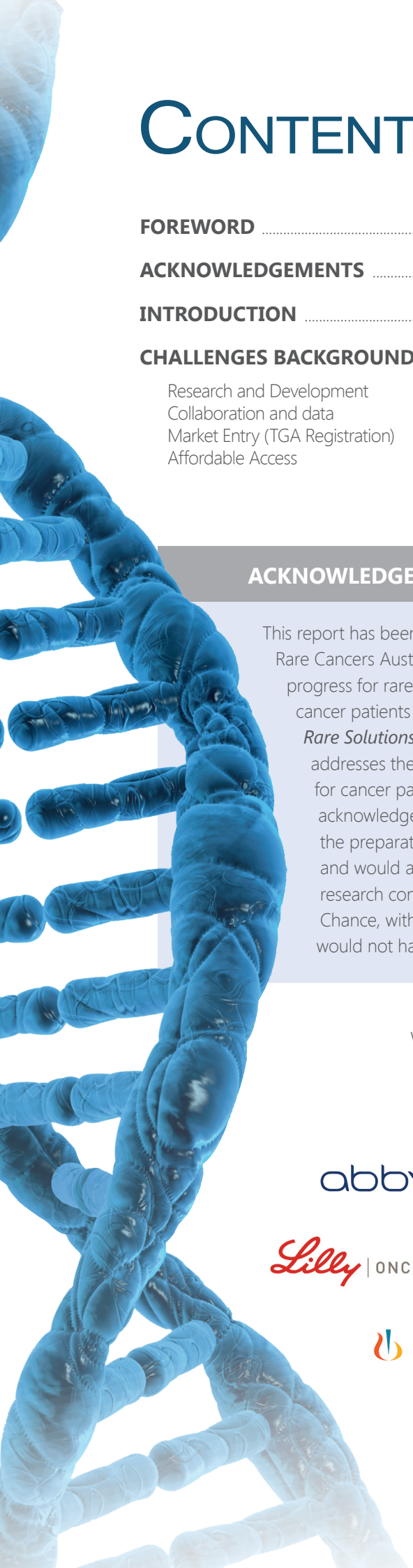
Collaboration and Real-World Data

Over the past months we have seen an increasingly strident argument around the introduction of My Health Record (MHR). There has been no balanced discussion appropriately weighing the enormous potential benefits of MHR with risk. In a world where health costs are a constant concern, people present at emergency with no clinical history, and cancer patients have sole responsibility of mountains of hard copy medical information, MHR's effective introduction is a potential life saver every day. Additionally, the economic benefits in tracking outcomes, avoiding duplications of service and providing much needed population level data are further reason to support this initiative and other collaborative and data management measures.

Summary

We have made great progress in the past year for the rare cancers patients of today and tomorrow. RCA is thankful to the Government and Minister Hunt in particular, as well as the patients, clinicians, public servants and industry representatives that continue to work hard to level the playing field for this group. There is more work to be done, but we are encouraged by the acknowledgement and determination shown and believe that Australia, as a caring society, can achieve parity of care for rare cancer patients in the coming years. For those impacted, it can't come soon enough.

Contents



ACKNOWLEDGEMENTS

This report has been prepared by Rare Cancers Australia (RCA) to show the progress for rare and less common (RLC) cancer patients since the publication of 2017's *Rare Solutions: A Time to Act*, and also addresses the implications of genomics for cancer patients. RCA gratefully acknowledges Eliza Mitchell's work in the preparation of this update report and would also like to acknowledge the research contribution made by Nash Chance, without whom this document would not have been possible.

This report draws on data publicly available from a number of organisations including the Australian Institute of Health and Welfare (AIHW), the National Health and Medical Research Council (NHRMC), the Medical Research Future Fund (MRFF), the Pharmaceutical Benefits Advisory Committee (PBAC) and the Therapeutic Goods Advisory (TGA). RCA would also like to acknowledge the patients and carers we work with in navigating our health system – their insights are pivotal in identifying areas of need where Australia must do better for those with RLC cancers.

We would like to also acknowledge those who have provided financial and in-kind support to RCA in the creation of this report. All support is gratefully received and has been given without pre-condition or editorial input.

abbvie · AMGEN · Bristol-Myers Squibb · Celgene · Lilly | ONCOLOGY · GILEAD · janssen · NOVARTIS ONCOLOGY · Pfizer Oncology · Roche

Many thanks also to Charlene Vien from RCA for her incredible work in the design and laying out of this report. In addition, we are grateful to Belynda Simpson of Bel-Art for her graphic design finesse.

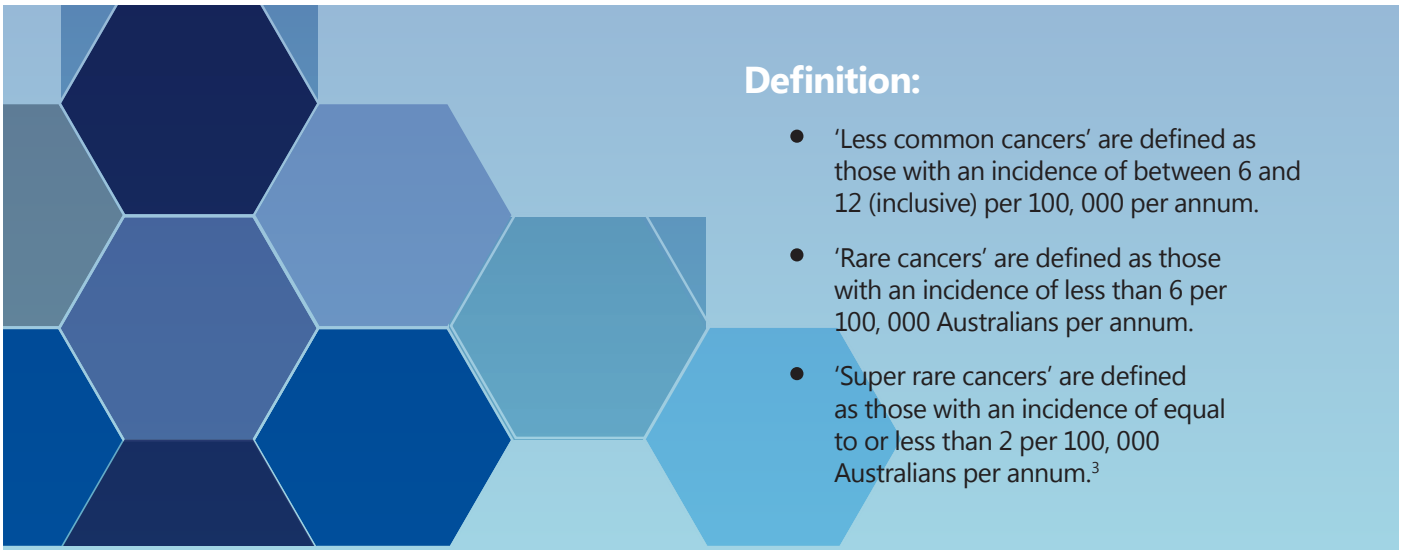
INTRODUCTION

Since its inception in 2012, Rare Cancers Australia (RCA) has helped to shine a light on rare and less common (RLC) cancer patients, who consistently fall through the cracks of research funding, early diagnosis, and access to subsidised cancer treatments. These factors have created the perfect storm, where RLC cancer patients as an aggregate have poorer survival outcomes, and indeed less hope, than their common counterparts.

The *'Just a Little More Time: Baseline Report'* and *'Just a Little More Time: Rare Cancers Update Report'* both highlighted the disparity in outcomes and showed the size of the problem for RLC cancer patients in Australia. The figures have grown with population, and it is estimated that in 2018 there will be 52,000 annual diagnoses of RLC cancers, and 25, 000 annual deaths. Despite now making up 38% of all cancer diagnoses, RLC cancers account for a disproportionate 52% of all cancer deaths.[1]

After highlighting this problem, RCA produced a report in 2017 aimed at providing solutions. *Rare Solutions: A Time to Act* brought together stakeholders from government, patient advocacy, industry and research to provide feasible recommendations aimed at remedying the significant disparities faced by RLC cancer patients.[2] These consecutive reports, initiated action and helped to establish the collective will for change. This *Rare Solutions: Progress Update* has been prepared to galvanise those recommendations. It examines the key elements; clinical trial structure and funding for RLC cancer patients, access to medicines through registered and reimbursed pathways, collaboration and data collection, and also highlights the need to be prepared for genomic technologies in cancer care.

RCA acknowledges the great work by patients, advocates, elected representatives, departmental policymakers and industry in beginning to address the challenges presented by RLC cancers. Patients are increasingly heartened by the ground-breaking research and work that gives them hope. This report is about making sure this hope is not out of reach because of the rarity of their disease, the size of their bank balance, or the speed of change.



Definition:

- 'Less common cancers' are defined as those with an incidence of between 6 and 12 (inclusive) per 100, 000 per annum.
- 'Rare cancers' are defined as those with an incidence of less than 6 per 100, 000 Australians per annum.
- 'Super rare cancers' are defined as those with an incidence of equal to or less than 2 per 100, 000 Australians per annum.[3]

1 Australian Institute of Health and Welfare. Cancer Compendium: Information and Trends by Cancer Type. 2018. Retrieved from https://bit.ly/2MY844J

2 Rare Cancers Australia. 2017. Rare Solutions: A Time to Act. Retrieved from https://bit.ly/2uBrXq4

3 Gatta G, van der Zwan JM, Casali PG, Siesling S, Dei Tos AP, Kunkler I, et al. Rare cancers are not so rare: the rare cancer burden in Europe. Eur J Cancer. 2011;47:2493-511.

CHALLENGES BACKGROUND

Australia can be thankful for some of the most robust regulatory and reimbursement systems in the world through its Therapeutic Goods Administration (TGA), the Medicare Benefits Schedule (MBS) and the Pharmaceutical Benefits Scheme (PBS). Together, the remit and purpose of these bodies has been to provide safe, universal, affordable and timely access to necessary medicines and devices for all Australians. However, the speed, volume and cost of newly developed technologies including innovative treatments such as immunotherapies and targeted medicines, have presented challenges to the system's aim of fairness, timeliness and availability.

The challenges of RLC cancers centre around four main areas; research and development, collaboration and data collection, registration, and reimbursement.

Research and Development

Small patient populations for RLC cancers mean that gold standard randomised clinical trials are difficult and often uneconomic to conduct. The patient numbers mean the standard of evidence for larger population groups cannot be easily replicated and there is little financial incentive for the high research costs. In many cases, the absence of credible existing treatments also creates challenges in providing a control arm in these trials.

Collaboration and Data

There are well over 200 rare and less common cancers, and over 800 rare subtype cancers.[4] Therefore, it is not uncommon to encounter patients who have heard phrases like 'I have never seen this kind of cancer before' from their clinicians. Education around diagnostics and referral for each of these individual cancers therefore is immensely challenging. Additionally, information around centres of excellence, compassionate access/ co-pay schemes and relevant clinical trials is not easily navigable, resulting in inconsistent treatment pathways and outcomes, and isolation for patients. Collaboration and information sharing are vital in addressing this challenge.

Market Entry (TGA Registration)

The aforementioned barriers to research and data collection create challenges at the point of market entry onto the Register of Therapeutic Goods (RTG) via the TGA. There has been commendable policy progress in this area via the Medicines and Medical Devices Regulation (MMDR) Review that addresses some of the issues for RLC cancers. It is essential that the opportunities provided by this change are taken advantage of, as the off-label use of new cancer medicines can result in immense inequity where economic standing decides treatment, sometimes referred to as 'survival of the richest'.

Affordable Access

Finally, cost is the largest barrier to treatment for RLC cancer patients. The Pharmaceutical Benefits Advisory Committee (PBAC) recommends medicines for reimbursement according to an assessment of their cost-effectiveness. Cost-effectiveness is more difficult to ascertain than clinical effectiveness for RLC cancer patients. This is because of the quality of data discussed above, and the degree of difficulty of these requirements. When industry and government cannot agree on price for clinically effective medicines, it is patients who miss out. Further comment on the progress and challenges of registration and reimbursement can be found on p.7.

Community concerns over access, equity and research in cancer are highlighted by the over 500 submissions to two Senate Inquiries in the last three years alone. The recommendations tabled from the *Inquiry into the Availability of New, Innovative and Specialist Cancer Drugs in Australia (2015),*[5] and the *Inquiry into Funding for Research into Low Survival Rate Cancers (2017)*[6] align with community will and the *Rare Solutions* proffered last year and within.

Rare Solutions and the Senate Inquiries above provide a roadmap for positive change, of which initial steps have been taken. RCA encourages a continuation of this trend to even the playing field for RLC cancer patients.

4 Gatta G, van der Zwan JM, Casali PG, Siesling S, Dei Tos AP, Kunkler I, et al. Rare cancers are not so rare: the rare cancer burden in Europe. Eur J Cancer. 2011;47:2493-511.

5 Senate Community Affairs References Committee. 2015. 'Availability of New, Innovative and Specialist Cancer Drugs in Australia. Retrieved from https://bit.ly/2onXXZ7

6 Senate Select Committee. 2017. 'Funding for Research into Cancers with Low Survival Rates'. Retrieved from https://bit.ly/2PkrLRY

PROGRESS - RECOMMENDATION 1

Part 1 – Clinical Trial Funding and Structure

Rare Solutions Recommendation 1:
Local clinical trials should be designed to support future TGA registration and PBS listing for rare cancer indications

1. Evidence for future PBS listing to be considered as one of the key outcomes for investigator led clinical trial design
2. Local investigator led clinical trials to have more flexible inclusion criteria, without compromising patient safety
3. Additional funding to support local clinical trials for RLC cancers
4. Global clinical trials should be expanded to include more rare cancer patients.[7]

Importance of Research

It is widely recognised that funding for research and clinical trials is essential to improving outcomes for patients. As discussed on p. 5, the nature of rarity, registration and reimbursement has traditionally precluded incentives for research and development in RLC cancers. The reduction in mortality for cancers such as breast and cervical highlight the positive return on strategic investment in research for both prevention and treatment of cancer.[8] Therefore, *Rare Solutions Recommendation 1* [Box 1] targeted the structure and incentivisation of clinical trials *as well as* total funding.[9]

Progress Past 12 Months

This progress update happily reports unprecedented public funding commitments in the past year to research for RLC cancers with high unmet need. Although there are various bodies, private and public, that fund cancer research, the most notable commitments this year have occurred via public funding through the Medical Research Future Fund (MRFF) and the Genomic Cancer Medicine Program (GCMP). Both of which begin to move beyond our traditional histological classification of cancers and into more molecular targeted classifications. Some structural barriers for NHMRC funding of RLC cancers identified in the 2017 Senate Inquiry[10] have meant that funding via this mechanism is not currently commensurate with burden of disease (51.7% of total cancer burden, with 8.2% of total NHMRC cancer funding).[11]

However, affirmative action via the other public funding mechanisms shows a necessary trend towards matching RLC research funding with burden of disease.

- $206 million commitment over five years to the Rare Cancers, Rare Disease and Unmet Need Clinical Trials program
- $50 million over 5 years for the expansion of the Genomic Cancer Medicine Program to eight centres of excellence across Australia
- $41 million NHMRC funding spent in 2017 on RLC cancers

This research, while not only saving the health system down the line with reduced acute care costs and greater productivity, is structured in a way to maximise options for funded treatment for RLC patients who have few subsidised options, providing hope for those who need it most.

7 Rare Cancers Australia. 2017. *'Rare Solutions: A Time to Act'*. p. 16-17. Retrieved from https://bit.ly/2uBrXq4

8 Rare Cancers Australia. 2016. *'Just a Little More Time: Rare Cancers Update Report'*. Retrieved from: http://bit.ly/JaLMT-2

9 Rare Cancers Australia. 2017. *'Rare Solutions: A Time to Act'*. p. 7. Retrieved from https://bit.ly/2uBrXq4

10 Senate Select Committee. 2017. *'Funding for Research into Cancers with Low Survival Rates'*, p. 6-7. Retrieved from https://bit.ly/2PkrLRY

11 National Health and Medical Research Council. Outcomes of Funding Rounds. 2018. Retrieved from https://bit.ly/2NOzXtc

PROGRESS - RECOMMENDATION 2

Part 2 – Flexible Access Models

Rare Solutions Recommendation 2: Use flexible approaches within existing frameworks to gain access to subsidised medications for super rare cancers

1. Pharmaceutical companies to explore and capitalise on flexibility within existing frameworks (e.g. managed access or risk sharing) to proactively seek registration and reimbursement for rare indications
2. PBAC to consider provisional (conditional) listing of medicines for rare cancer indications where appropriate
3. New targeted therapy submissions to the TGA and PBAC should include rare indications using a multi-indication submission[12]

Why Do We Need Flexibility?

Advancements in research mean that countless innovative oncology medicines are being trialled across multiple cancer types. There are approximately 800 cancer medicines currently in industry research pipelines,[13] however, the model for the discovery, development and reimbursement of treatments has not traditionally been suitable for RLC cancers.[14] There are many factors that can lead to delays for RLC cancer listings including; lack of TGA registration, lack of PBAC applications and approvals, the comparatively higher price of these medicines than current alternatives, and the resource intensiveness for industry, the PBAC and government in producing and assessing applications and listing medicines (in addition to the barriers mentioned on p. 5). Additionally, the TGA and the PBAC are restricted in their ability to initiate applications for registration and reimbursement respectively, so this is reliant on the sponsor.

Progress Past 12 Months

- TGA reform via expedited 'Priority Review' and 'Provisional Approval' pathways
- PBAC inquiry into subsidy consideration for multiple cancer types

TGA Flexibility There has been significant progress made for expedited TGA registration pathways in the past year. The Medicines and Medical Devices Regulation (MMDR) Review resulted in;

- **Priority Review Pathway (July 2017) –** This involves faster assessment of medicines where there is a full data dossier provided – with a target timeframe of 150 days for eligible serious and life-threatening conditions.
  - **7** successful priority designations for RLC cancer indications have been achieved[15]
- **Provisional Approval Pathway (March 2018)** – This results in earlier access to promising new medicines that do not yet have a full data dossier, but where there is the potential for substantial benefit to Australian's through earlier availability
  - **3** successful provisional designations have been achieved[16]

Where the Priority Review pathway may not be feasible because of only early clinical trial data or small patient populations, we encourage pharmaceutical companies to utilise the Provisional Approval pathway wherever possible to ensure RLC cancer patients have timely access to registered cancer medicines.

Orphan drug designation – TO BE ADDRESSED
Orphan drug designation is a process where sponsors receive a fee waiver to help bring medicines for a small population to market. It has been a great initiative, but recent changes threaten its feasibility. We recommend an 18-month fee waiver period with indefinite waiver if a PBAC application is ongoing. The designation needs to ensure sponsors are incentivised to act quickly, whilst still providing a practical timeframe to do so.

PBAC Flexibility

On the reimbursement side of the access issue, there has been progress via an inquiry into PBAC assessment flexibility – whereby medicines may be able to be assessed for multiple indications at one time.

At the time of writing, the PBAC is currently considering options for subsidy consideration for multiple cancer types (for PD-1 and PD-L1 checkpoint inhibitor immunotherapies). The result of this inquiry is unknown at the time of publication, but RCA endorses a solution that offers provisional listing of medicines via a managed access model for RLC cancer patients, as in *Rare Solutions Recommendation 2* [Box 2]. This would begin to address the financial and access inequities faced by RLC cancer patients.[17]

12 Rare Cancers Australia. 2017. 'Rare Solutions: A Time to Act'. p. 22-23. Retrieved from https://bit.ly/2uBrXq4

13 Deloitte Access Economics. 2017. 'A Collaborative Assessment of Access to Cancer Medicines in Australia', p. 3. Retrieved from https://bit.ly/2rp2luP

14 Rare Cancers Australia. 2017. 'Rare Solutions: A Time to Act'. p. 11. Retrieved from https://bit.ly/2uBrXq4

15 Therapeutic Goods Administration. 2018. 'Prescription medicines determination and designation notices.' Retrieved from https://bit.ly/2BWTu9u

16 Ibid

17 National Oncology Alliance Submission to PBAC August Special Meeting. 2018. 'PD-1 and PD-L1 checkpoint inhibitor immunotherapies: options for subsidy consideration for multiple cancer types'. Retrieved from https://bit.ly/2PH938d

PROGRESS – RECOMMENDATION 3

Part 3 – Collaboration and Data

Recommendation 3:
Improve structured collaboration to provide consistency and standards for all stakeholders

1. Australia to have an appropriate national framework that provides leadership, oversight and support to Australians living with an RLC cancer
2. Patients to have a consistent and equitable experience that also supports the development of further evidence for future patients
3. Australia to have a nationally consistent approach to collect outcomes data for all patients with RLC cancers.[18]

For RLC cancer patients and their clinicians, information is critical. For people who may only have a few months to live, not being able to find the right specialist or experimental treatment in time can be extremely costly. *Rare Solutions Recommendation 3* [Box 3] urged for collaboration between all stakeholders in the cancer community to address the issues RLC cancer patients face in navigating a complex health system. In conjunction with stakeholder collaboration, consistent approaches to outcomes data collection will improve consistency of care and empower both RLC cancer patients and their clinicians.

Rare Solutions Recommendations 1 + 2 both involve collection of outcomes data. My Health Record (MHR), with improved functionality, is an ideal tool for the management of nationally consistent data collection. For instance, the adoption of managed access programs with real world evidence collection for RLC cancers (via MHR) would improve flexibility of reimbursement and thus timeliness and affordability of treatment for patients. Data is at once both more difficult to come by for RLC cancer patients, and essential to ensuring patients have access to research and treatment. It is critical that the approach to data collection and sharing is nationally consistent and agreed upon by all relevant stakeholders to ensure best outcomes for patients.

18 Rare Cancers Australia. 2017. 'Rare Solutions: A Time to Act'. p. 26-27. Retrieved from https://bit.ly/2uBrXq4

Progress Past 12 Months

- $42 million new funding over five years via the MRFF to support international clinical trial collaborations
- Launch of the National Oncology Alliance (NOA), bringing together patients and carers, industry, patient organisations and clinicians
- $374.2 million projected spend on MHR over the next two financial years[19]
- Launch of the online 'KnowledgeBase' platform with the support of Cancer Australia [see Box. 1][20]

My Health Record

The electronic storage and transfer of health records is essential to healthcare delivery in the 21st century. As with any large-scale health initiative like Medicare or the NDIS, we need to reasonably calculate and minimise the risks and weigh these against the potential benefits, particularly to patients and the healthcare professionals responsible for their care.

Keeping track of medical information can be difficult, especially in cases of cancer or other acute illnesses where the breadth of information and the number of involved healthcare professionals drastically increases. MHR will provide those entrusted with looking after patients, a more complete picture. That being said, there has been some criticism around the practicality of a *document-based* eHealth system; it is critical that the MHR is functional and fit-for-purpose for both healthcare professionals and patients.[20] 12% of hospital admissions in Australia are estimated to be medication-related, increasing to over 20-30% for people over 65 years.[21] Although there have been concerns around security of data, it is critical that we don't allow fear to make us lose sight of the benefits of shared information for consistency of care. Indeed, respondents in the MHR participation trials 'expected that their healthcare providers [were already] sharing information with other healthcare providers in this way'.[22] Additionally, the upcoming inquiry into MHR will provide great opportunity for community consultation around the functionality and security of the initiative.

19 Department of Health. 2018. 'My Health Record – continuation and expansion'. Retrieved from https://bit.ly/2uBrXq4

20 Rare Cancers Australia. 2018. 'About the Rare Cancers Australia KnowledgeBase'. Retrieved from https://bit.ly/2wpXpX4

21 Minion, L. 2017. 'GPs and hospitals claim My Health Record is not fit for purpose as alarmingly low usage figures are released' Retrieved from https://bit.ly/2PUDwzM

22 Roughead, L., Semple, S., & Rosenfeld, E. 2013. 'Literature review: medication safety in Australia'. Australian Commission on Safety and Quality in Health Care.

CONCLUSION

Rare and less common (RLC) cancers, by their very nature, exist in a challenging environment for research and access to subsidised treatment. They account for 38% of all cancer diagnoses and have increased to account for approximately 52% of all cancer deaths. Last year, RCA produced *Rare Solutions: A Time to Act* to provide feasible recommendations aimed at remedying the significant disparities faced by RLC cancer patients. The report recommended action around; the structure, incentivisation and funding of clinical trial activity for RLC cancers, flexibility of registration and reimbursement processes, and collaboration in research and data collection. This *Rare Solutions: Progress Update* reports that although there is still a way to go for RLC cancer patients, the past 12 months has seen positive action by stakeholders across all three recommendations.

Recommendation 1: Local clinical trials should be designed to support future TGA registration and PBS listing for rare cancer indications.

The structure, incentivisation and funding of clinical trial activity for RLC cancer patients has received a boost with;

- $206 million commitment over five years to the Rare Cancers, Rare Disease and Unmet Need Clinical Trials program
- $50 million over 5 years for the expansion of the Genomics Cancer Medicine Program to eight centres of excellence across Australia
- $41 million NHMRC funding spent in 2017 on RLC cancers

Recommendation 2: Use flexible approaches within existing frameworks to gain access to subsidised medications for super rare cancers.

There have been moves towards greater flexibility in registration and reimbursement by both the Therapeutic Goods Administration (TGA) and the Pharmaceutical Benefits Advisory Committee (PBAC);

- TGA reform via expedited 'Priority Review' and 'Provisional Approval' pathways
- PBAC inquiry into subsidy consideration for multiple cancer types

With regard to Recommendation 2, RCA encourages greater uptake of the TGA's 'Provisional Approval' pathway by the pharmaceutical industry. In addition, RCA requests a review of the feasibility of the new 6-month Orphan Drug Designation fee waiver and suggests an 18-month waiver period, with indefinite waiver where there is an ongoing PBAC application.

Recommendation 3: Improve structured collaboration to provide consistency and standards for all stakeholders.

Cancer community collaboration, as well as consistent outcomes data collection is essential to buttressing Recommendations 1 + 2, and ensuring equitable access to research and treatments for RLC cancer patients

- $42 million new funding over five years via the MRFF to support international clinical trial collaborations
- Launch of the National Oncology Alliance (NOA) bringing together stakeholders including patients and carers, industry, patient organisations and clinicians
- $374.2 million projected spend on MHR over the next two financial years
- Launch of the online 'KnowledgeBase' platform by RCA with the support of Cancer Australia

Genomic research is presenting wonderful opportunities for RLC cancer patients and their carers [Appendix A]. Due to our evolving understanding of the molecular drivers of cancer, research into rare tumours is now more accessible than ever before. These great medical discoveries have been matched by an ambitious strategic funding plan via the Australian Genomics Mission, and it is essential the regulatory environment matches the ambitions for wholesale adoption of genomics and precision medicine.

The various innovative approaches to policy, increased funding for research and genomics, and greater collaboration between stakeholders in the cancer community show that the voices of RLC cancer patients are starting to be heard. RCA will continue to work with patients and carers, government, researchers, industry and other stakeholders to even the playing field for those who need it most, and to ensure the *Rare Solutions* within become a reality.

APPENDIX A

Genomics Opportunities and Preparedness

Genomics and precision medicine 'is the future for all cancer treatment',[23] and provides opportunity for patients to be treated on the genetic determinants of their disease, rather than where in the body their cancer has occurred. This is extremely encouraging for RLC cancer patients, where small patient populations have traditionally disadvantaged access to research and clinical trials, and in turn, registered and reimbursed treatment pathways, resulting in poorer outcomes for this group.

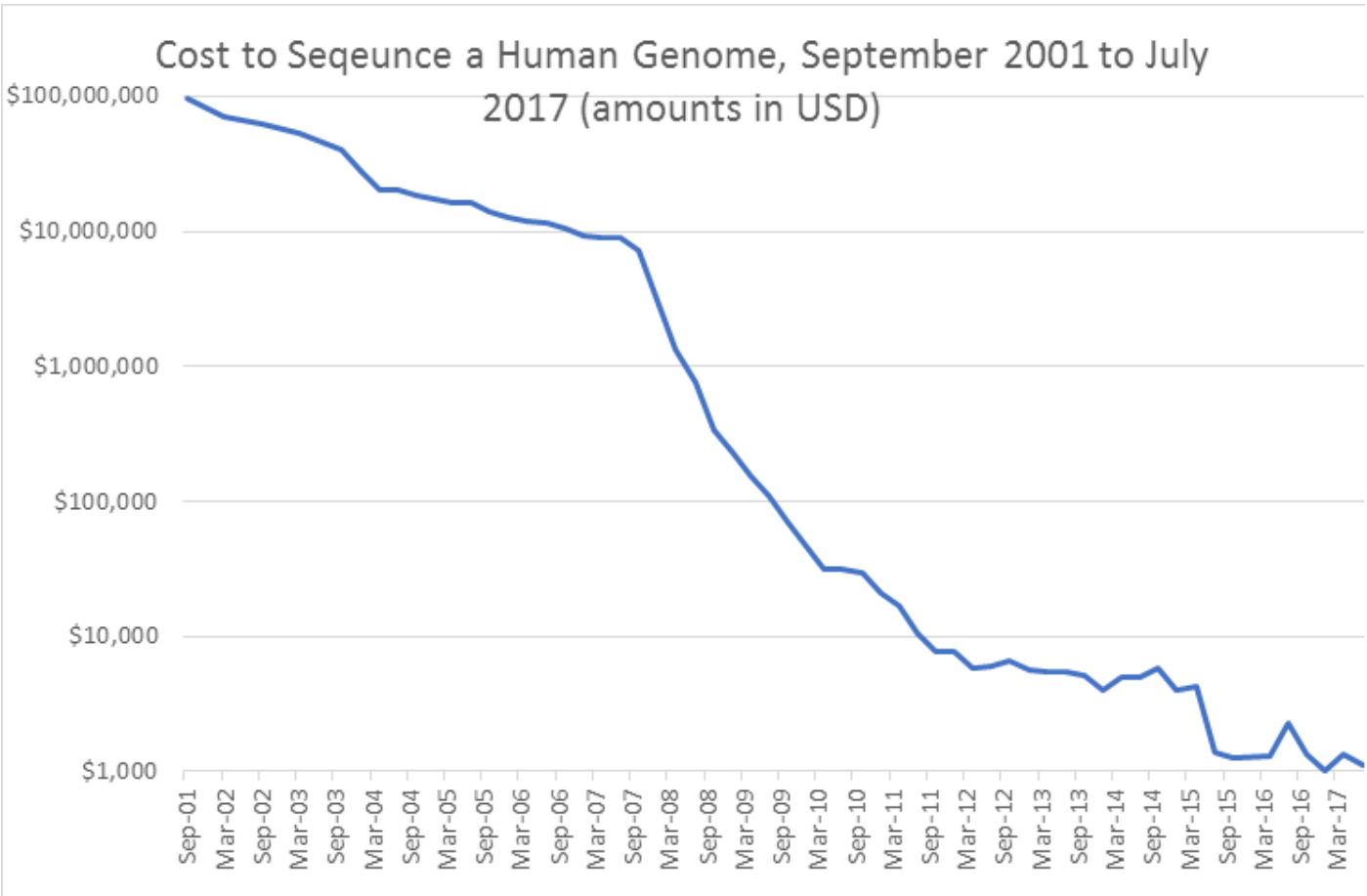
A very welcome disruptor in health, genomics and precision medicines' move towards greater integration into mainstream cancer care, will continue to be facilitated by great reductions in sequencing costs [see Fig. 1].

2017 saw a $500 million, 10-year federal government commitment to an Australian Genomics Mission, as well as recognition by Innovation and Science Australia of genomics and precision medicine as a strategic opportunity in the Australia: 2030 plan, highlighting the large role genomics will play in public health in the 21st century.[24]

RLC cancer patients will be able to see the benefits of genomics and precision medicine through;

- Earlier and more accurate diagnosis – a greater range of targeted, cost-effective population genome screening programs to identify RLC cancers

Fig. 1



23 Submission 34, Garvan Institute of Medical Research. 2017. Senate Select Committee, 'Funding for Research into Cancers with Low Survival Rates'. Retrieved from https://bit.ly/2PkrLRY

24 Department of Health. 2018. National Health and Medical Industry Growth Plan – Australian Genomics Health Futures Mission. Retrieved from https://bit.ly/2lqTkcv

- Prevention – increased awareness of cancer risk, targeted screening and self-management of lifestyle and prevention activities
- Better and safer treatments – individual drug and treatment matching, enabled by integration of genomic data, gene therapy and gene editing.[25] [26]

RCA is delighted by the investment and research in genomics and notes that as with other research, it is essential that barriers to patient participation and benefits are reduced as far as practicable to encourage better health outcomes.

One barrier that has arisen is around perceived or actual genetic discrimination faced by consumers when obtaining life, disability and trauma insurance. A recent inquiry into the Life Insurance Industry by the Parliamentary Joint Committee into Corporations and Financial Services, raised some issues around this third-party use of genetic information.[27] Evidence provided to the committee showed several concerns around the industry's current, not legally binding, regime of 'self-regulation'. Similar health system examples such as Canada and the UK have introduced measures around the use of genetic information. The UK has adopted a Concordat and Moratorium, and Canada legislated the *Genetic Non-Discrimination Act*, which amongst other measures, prohibits insurers from requesting disclosure of previous or future genetic test results. In line with the committee, RCA is also 'concerned about evidence received that individuals are not undertaking potentially life-saving genetic testing due to fears of unfair treatment by life insurers'.[28] The committee also expressed concern over the 'use or perceived use of genetic information by life insurers [impacting] on participation in public health research projects and other forms of research'.[29]

The benefits of earlier and more accurate diagnosis, prevention, and better and safer treatments will most immediately and most importantly be felt by RLC patients and carers. In addition, there will also be obvious economic and productivity benefits to the taxpayer and wider community. Research provides hope and better outcomes for RLC cancer patients, and it is critical that Australia has a policy and regulatory environment that promotes patient confidence in evolving genetic testing. RCA commends the recent strategic and financial investments particularly in genomic research and looks forward to working with other stakeholders in the community to ensure Australia can sufficiently take advantage of the exciting opportunities presented by genomic research.

25 Innovation and Science Australia. 2017. 'Australia 2030: prosperity through innovation'. Retrieved from https://bit.ly/2NwRlmo

26 Submission 34, Garvan Institute of Medical Research. 2017. Senate Select Committee, 'Funding for Research into Cancers with Low Survival Rates'. Retrieved from https://bit.ly/2PkrLRY

27 Parliamentary Joint Committee on Corporations and Financial Services. 2018. 'Life Insurance Industry' Retrieved from https://bit.ly/2pH6uHB

28 Ibid, p. 155

29 Ibid p. 154-155

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