

People living with cancer urgently need action on expanded access to precision therapies

Over the past two decades, our ability to rapidly obtain genomic information on an individual and their cancer has led to a dramatic shift in how we can diagnose, treat, monitor and prevent cancer, leading to an era of precision oncology.

With this expanded knowledge of cancer there are several promising treatments that can specifically target the drivers of an individual's cancer, that are already under review through Australia's health technology assessment process. Too many people living with cancer in Australia still don't have access; and they can't afford to wait.



Precision therapies provide significant benefits for people living with cancer

Knowledge of the molecular mechanisms that drive cancer and progression has facilitated the creation of a range of new therapies that either target specific cancer cells or manipulate our own immune system to attack the cancer cells. Unlike traditional chemotherapy drugs, these therapies can specifically target cancer cells while avoiding damaging normal cells.

Due to their precise nature, these therapies often produce better responses and are less toxic. For example, larotrectinib, a highly selective tropomyosin receptor kinases (TRK) inhibitor, has been shown to deliver marked and durable responses (86% response rate) in people with TRK fusion-positive cancers, regardless of the age of the patient or of the tumour type.¹

Collectively, this has enabled a shift from the traditional one-size-fits-all approach based on the location of cancer to a new era of precision medicine, where treatment is based on the genomic make up of each person's cancer.



Access for Australian patients is lagging behind other countries

In Australia, our health technology assessment (HTA) system assesses and approves cancer therapies on an indication-by-indication basis, one at a time. As a result, people with cancers of the most common indications receive access to precision therapies first, while others with rarer indications continue to wait. We can do things differently.

Perhaps most importantly for improving access to precision therapies, with some landmark approvals in 2017 and 2018, we are now seeing pan-cancer, tissue-agnostic therapies being approved overseas. For example:

- Pembrolizumab was approved by the FDA, in 2017, for people with tumours characterised as MSI-H/dMMR.²
- Larotrectinib and entrectinib were also FDA approved in 2018 and 2019 for advanced solid tumours (independent of anatomic location of the tumour i.e. tumour-agnostic) with NTRK fusions.^{3,4}

Since then, a total of five molecular biomarkers, including BRAFV600E mutations, RET fusions, NTRK fusions, high tumour mutation burden (TMB), and dMMR/MSI-H, have obtained tissue-agnostic approvals from the US Food and Drug Administration (FDA).⁵ Meaning more people get access faster, meanwhile people living with cancer in Australia continue to wait.

The PD-1/PD-L1 inhibitor pembrolizumab was first available to Australian patients in September 2015 when it was listed on the Pharmaceutical Benefits Scheme (PBS) for people with metastatic melanoma. Today, there have been seven PD-1/PD-L1 inhibitors approved by the FDA for a range of haematological cancers and solid tumours, namely nivolumab, atezolizumab, avelumab, durvalumab, cemiplimab, dostarlimab and most recently, retifanlimab.⁶

While pembrolizumab is now publicly funded for 12 types of cancer and there are currently around 3,500 Australians receiving it through the PBS, we estimate that:

At least

3,500 more people


would be eligible for treatment with a PD-1/PD-L1 inhibitor if PBS funding was available for all indications that are currently approved in the US.


Around


7,000 more people

will be eligible once the remaining indications are eventually approved.⁶

Contact Us

 (02) 4862 2768

 Christine@rarecancers.org.au

 PO Box 440
Bowral NSW 2576



It's time for people to have access to the precision therapies they need

Precision oncology approvals are crucial to accelerating access for people with cancer. This is particularly important for those living with the rarest cancers who may never have access to effective therapies because of small patient populations and the challenges with meeting evidentiary requirements in HTA.

It is time for Australia to realise the full potential of precision oncology and deliver equitable outcomes across all cancer types. **To do so, we urge stakeholders to show leadership and work together to recognise the potential for improving the lives of people living with cancer and their families.**

It is crucial that our HTA system delivers the best technologies and treatments to people without delay. While we acknowledge the HTA review, which has identified the need for guidance on how to address tumour-agnostic approvals, people living with cancer can't afford to wait. They need us to act and ensure they can access the treatments they need before it's too late.



Supported by:

Prof David Thomas

Director
Centre for Molecular Oncology
School of Biomedical Sciences
University of New South Wales

A/Prof. Arun Azad

Medical Oncologist
Peter MacCallum Cancer Centre
Melbourne

Dr Matthew Burge

Medical Oncologist
Mater Hospital, Rockhampton

Prof Clare L Scott AM

Medical Oncologist
Peter MacCallum Cancer Centre
Royal Melbourne and
Royal Women's Hospitals

A/Professor Aaron R. Hansen

Deputy Director
Senior Medical Oncologist
Division of Cancer Services
Metro South Health

Prof Tim Price

Clinical Director
Bragg Comprehensive
Cancer Centre
South Australia

Professor Declan G Murphy

Director of
Genitourinary Oncology
Peter MacCallum Cancer Centre
Melbourne

Prof Howard Gurney

Director of Clinical
Trials and Clinical Program
Head, Oncology
Macquarie University

Professor John R Zalberg AO

Consultant Medical Oncologist
Alfred Health, Epworth
Hospital and Cabrini Health

1. Drilon A, Laetsch TW, Kummar S, et al. Efficacy of Larotrectinib in TRK Fusion-Positive Cancers in Adults and Children. *N Engl J Med* 2018; 378: 731-739. DOI: 10.1056/NEJMoa1714448.
2. Food and Drug Administration. FDA grants accelerated approval to pembrolizumab for first tissue/site agnostic indication, <https://www.fda.gov/drugs/resources-information-approved-drugs/fda-grants-accelerated-approval-pembrolizumab-first-tissuesite-agnostic-indication> (accessed 20/03/2024).
3. Food and Drug Administration. FDA approves larotrectinib for solid tumors with NTRK gene fusions, <https://www.fda.gov/drugs/fda-approves-larotrectinib-solid-tumors-ntrk-gene-fusions> (accessed 20/03/2024).
4. Dunn DB. Larotrectinib and Entrectinib: TRK Inhibitors for the Treatment of Pediatric and Adult Patients With NTRK Gene Fusion. *J Adv Pract Oncol* 2020; 11: 418-423. 20200501. DOI: 10.6004/jadpro.2020.11.4.9.
5. Gouda MA, Nelson BE, Buschhorn L, et al. Tumor-Agnostic Precision Medicine from the AACR GENIE Database: Clinical Implications. *Clin Cancer Res* 2023; 29: 2753-2760. DOI: 10.1158/1078-0432.Ccr-23-0090.
6. Rare Cancers Australia. Rarefication - personalised medicine in the genomic revolution, https://rcararecancers.blob.core.windows.net/assets/contentpage_htmlcontent/Rarefication%20FA%20DIGITAL%20r3.pdf (2023, accessed 07/03/2024).