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It's hard to test therapies for rare cancers because there are too few people to study. from www.shutterstock.com.au

Rare cancers are just that: rare. This means research into each of these particular types of rare cancers is limited, and so are the treatment options. As a consequence, patients diagnosed with rare cancers face significant challenges.

In November 2016, the Australian Senate established a select committee to examine funding for research into cancers with low survival rates. More recently, the health minister announced A$13 million from the Medical Research Future Fund will be used for clinical trials to help achieve better health outcomes for people with rare or uncommon cancers.

The minister also commissioned new work on evaluating cancer medicines that treat multiple tumours and have a specific genetic feature (biological marker). This could improve access to therapies that might benefit some patients with rare cancers.

These recent steps are in recognition of the significant challenges associated with undertaking research into rare cancers. By their nature, rare cancers include small and variable patient populations making gold-standard randomised trials challenging or even impossible.

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The lack of evidence resulting from few or no randomised trials creates challenges for registering and reimbursing new medicines. This ultimately leads to a lack of subsidised medicines for these patients. As a result, the improvements seen in patient outcomes related to new therapies for more common cancers like lung cancer, melanoma and bowel cancer over

the last two decades do not extend to rare or less common cancers .

What is a rare cancer?

The definition of a rare cancer is debatable. The RARECARE collaboration in Europe uses an operational definition of fewer than six cases per year per 100,000 population. In Australia, the medicines regulator, the Therapeutic Goods Administration (TGA), has recently updated the eligibility criteria for medicines treating rare diseases to fewer than five cases of the disease in a population of 10,000 people.

Historically cancers were categorised by the anatomical location, such as the breast or kidney. But with the discovery of new biological markers, common cancers can be grouped into smaller, more homogeneous and genetically similar subsets. So the number of rare cancers will continue to grow as medical technology advances.

Why don't they have many available medications?

The lack of government-approved and subsidised medicines to treat rare cancers primarily stems from the lack of evidence supporting their use. Submissions to the current inquiry also cited problems such as a lack of research funding; the need for international collaboration; lack of investment by industry; attracting sufficient interest of researchers and recruiting sufficient patients.

It's hard to recruit enough patients for research studies.from www.shutterstock.com

Even if patients can be identified and recruited to a trial, it's difficult to generate meaningful data from so few patients.

The lack of evidence presents challenges for new medicines trying to meet registration and

reimbursement criteria in Australia. To be registered through the TGA, a new medicine must have demonstrated efficacy and safety.

In order for new medicines to be listed on the Pharmaceutical Benefits Scheme (PBS), it must have a demonstrated benefit over standard treatment, as well as being considered an efficient use of tax payer dollars
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New medicines for rare cancers are often expensive, especially when randomised trials are not possible.

What can we do to improve this situation?

With the changing nature of medicine and research, new opportunities are emerging to address the current inequity. The shift to treating patients based on the genetic profile of their tumour rather than the location of the cancer has increased treatment options for rare cancer patients.

Read more:** **How cancer doctors use personalised medicine to target variations unique to each tumour

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To harness the benefits, changes are required with input from multiple stakeholders, including government, industry, clinicians, researchers and patients.

Better access to new medicines ultimately starts with better research. To achieve this, experts have called for
additional targeted funding, innovative trial designs, and better partnerships between industry and researchers.

There is also the opportunity to collect better “real world” data via platforms such as the My Health Record , which could supplement existing research and allow performance monitoring of recently approved new medicines.

Organisations such as Rare Cancers Australia and the Cancer Drugs Alliance are liaising with government regarding changes that could improve access to novel medicines for patients with rare cancers. This includes greater input from patients and more flexibility in the way we evaluate medicines for public reimbursement.

It should also be recognised the problems faced in providing innovative treatments to patients with rare cancer extends to rare diseases in general . With modern medicine providing the potential to improve outcomes for patients with rare cancers as well as other serious chronic diseases, we need to have a broader conversation about what we can afford and what we are willing to pay for new medicines.

Colman Taylor is an owner and Director of Health Technology Analysts which provides consulting services to industry and Government. As part of this work, Colman Taylor has undertaken paid consulting work for manufacturers of rare cancer therapies. Neither Colman Taylor nor Health Technology Analysts has received remuneration in relation to the development of this article.

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Read more http://theconversation.com/how-we-can-overcome-the-lack-of-treatment-options-for-rare-cancers-83486