SIMPLIFY CANCER

Why Patients Are Key to Making Real World Evidence Work Faster

New Model for Better Access To Oncology Therapeutics in Australia

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Overview

Cancer is a leading cause of death in Australia. There is an unprecedented need for new medicines to help patients today. But bringing innovative therapeutics to market is a difficult task. It is a time consuming and costly exercise for biopharmaceutical companies who must demonstrate safety, efficacy and value for money. Regulators face challenges in having the right evidence to make timely decisions that improve public health.

Real-World Evidence (RWE) is a novel approach of using patient data to improve understanding of risks and benefits of novel therapeutics. This is an opportunity to improve our understanding of disease and build a stronger case for bringing innovative oncology medicines to the market. Real world evidence brings many benefits and opportunities including greater accuracy in reporting patient outcomes, improved decision support with hybrid trial designs and new, more flexible funding mechanisms.

The key stakeholders for using real-world evidence to improve health outcomes include biopharmaceutical industry, health technology assessment, healthcare professionals and patients within the broader community. Currently, patients are involved as a token gesture. There is no clear evidence of patient input into the regulatory decision making process.

In order for real world evidence to deliver on its promise, it needs to include medical expertise, expert analysis, regulatory guidance with patients at the heart of change as principal representatives of the wider community with lived experience and shared desire for change.

Meaningful patient engagement using the strategies outlined in this paper will help to bring innovative medicines to market faster and support the real world needs of those who need it the most:

• Advise and inspire our patients via a series of collaborative podcasts, video series, webinars and apps

• Unite patients as entrusted health activists in a single organisation that works closely with regulators and biopharma industry to lobby for novel therapeutics

• Bring patient perspective to the fore by establishing health based social network

The case for better health in a challenging world of today

According to Australian Institute of Health and Welfare, cancer is a leading cause of death in Australia – almost 50,000 deaths from cancer were estimated for 2020. And while the survival for more common cancers has improved [1], we can still expect that one in two Australian men and women will be diagnosed with cancer by the age of 85 [1].

We are facing an unprecedented demand for new cancer treatments. The innovation in oncology drugs is driven by the biopharmaceutical industry and the process to getting new medicines to help those need is a high-risk, high-cost undertaking. When we take into account the reality of the clinical trial success rates in oncology to be the lowest in the industry at nearly 3% [2] and the extended health technology assessment process (on average, new cancer medicine is listed on the Pharmaceutical Benefits Scheme (PBS) about two years after the initial submission [3]), cancer patients are facing significant delays in access to innovative therapeutics.

The cost for cancers among people in Australia diagnosed during 2009–2013 was approximately $6.3billion (0.4% of Gross Domestic Product; $272 per capita) [4]. With population growth and aging, these numbers can be expected to rise. To manage the escalating medical costs while improving health outcomes for our population, we must find better ways to expedite the process from drug discovery and clinical trials to regulatory review and patient care.

Real world opportunities for real world evidence

Real-World Evidence (RWE) is a way of using patient data from patient registries, medical records, health apps and social media to improve our understanding of risks and benefits of novel therapeutics. This approach can help to not only prove that the treatment is safe, effective and of a high standard, but also help to demonstrate that it's a sustainable, value for money investment. Real-World Evidence opens the way for us to bring better treatments to our patients, faster.

Greater accuracy in reporting patient outcomes

Randomised clinical trials have stringent eligibility criteria to determine who can take part. These criteria can exclude patients based on age, level of risk as well as prior treatments and medical conditions. As a result, those enrolled in randomised clinical trials can differ from real world population.

With real-world evidence, we can expand our understanding of the drug in clinical practice for patients who were underrepresented in — or completely excluded from clinical trials [5]. Using health records and patient surveys, we have an opportunity to gain greater insight into how effective the treatment is over time and may identify potential adverse events in special patient populations.

Improved decision support with hybrid trial designs

In 2018, the United States Food and Drug Administration published the framework to evaluate the potential use of real-world evidence (RWE) for regulatory purposes. The FDA framework suggests using hybrid design that integrates the traditional randomised clinical trial with pragmatic design aspects to collect real-world data on patients [6].

A hybrid trial could use Real-World Data for one clinical outcome such as a physical symptom, treatment toxicity, or psychosocial problem and more traditional elements such as specified entry criteria and monitoring. One key advantage of a hybrid design is that the data collected is representative of real-world health-seeking behaviour, medical practice, and outcomes. [7]

New, more flexible funding mechanisms

Real-World Evidence paves the way for assessing overall survival and patient experience in a real-world setting. This can make it easier for health technology assessment agencies including Therapeutic Goods Administration (TGA) to assess the value proposition of new medicines for cancer.

The assessment of real-world performance of cancer drugs paves the way for introducing new funding mechanisms such as conditional listing, outcomes-based managed entry or value-based pricing. Real-world evidence may also enable the renegotiation of drug prices to fund new cancer medicines [8].

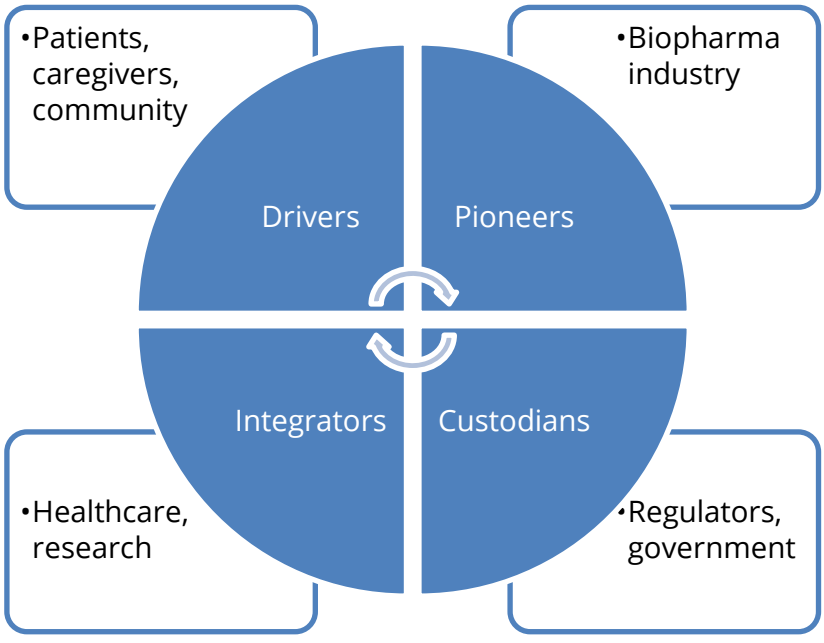
Patients as drivers for better health outcomes

Many groups share a commitment to improving health outcomes in cancer. From biopharmaceutical industry and regulatory bodies to not for profit groups, from researchers and clinicians to government organisations and private insurers, we are united in bringing forward the better care for cancer patients.

Current approach to patients from the regulatory perspective is often described as tokenistic [9]. Patients do not have a seat at the table of the decision makers because their input as viewed as perfunctory, their influence marginalised as a result.

Patients are not a divergent, eccentric troupe, but the community exemplar for health with the essential perspective of lived experience. No one is immune from cancer – the doctor, the regulatory specialist or the researcher can be a patient. This change does not skew or invalidate the person's perspective, but instead provides added value through the prism of living with the disease.

The following model suggests a symbiotic, community-driven approach with patients at the heart of change:



Our ambition to improve health outcomes for those with cancer begins with the patient. The needs we have as patients reflect the depth of our experience and the insights it can provide to the biopharma to drive innovation. The biopharmaceutical industry, despite the remarkable achievements in tackling disease in the modern age, do not get the recognition they deserve.

COVID-19 pandemic has brought to light the fear, confusion and misapprehension that some in our community share about the modern medicine and the vital role that biopharmaceutical industry has. We must bring down the many myths and misconceptions that exist in our society and restore our trust in science.

This can be achieved by forging stronger bonds with our government and regulatory bodies, the custodians of our welfare and the common good. Us as patients have a duty to share our perspective with those custodians in a way that brings forward the unified and distinct take on the true value of novel therapeutics for treating cancer.

The promise of improving health of our patients is empty without the recognition of the Australian healthcare community. They are the integrators of health and science who bring critical care to the patient. As patients, we are humbled and grateful to our doctors, nurses, researchers and healthcare workers by their incredible efforts to help those of us who are dealing with cancer.

Now is the opportunity to stand against misinformation and the many challenges we face in the wake of the global pandemic and make good on the potential of real-world evidence and patient reported outcomes to discover new insight on the true depth of the patient experience.

Advise and inspire our patients

Patients should not be viewed as a passive recipient of care or a token participant, but as the crucial link between the pioneers (biopharma), the custodians (regulators and government) and the integrators (healthcare community). There is an assertion separating patients from the broader community. This cannot be accepted as true – patients are principal representatives of our community with lived experience.

Many patients are not privy to the issues and circumstances that affect them and their loved ones. We need to advise and inspire our patients on the key issues surrounding access to cancer medicine and better living beyond cancer, highlighting the key role of the biopharmaceutical industry in advancing public health.

This can be done via a podcast and/or video series that discussing the innovative therapeutics, key challenges in bringing innovative therapeutics to market. These can take the form of interview and panel discussions with subject matter experts from across the spectrum.

Patient engagement needs to be patient driven because of the shared experience and common struggle through a range of innovative collaborations between private companies, public organisation and patient champions.

Unite patients as entrusted health activists

A patient led group can unite patient to advocate for the shared perspective. Working closely with Medicines Australia, biopharmaceutical industry, leading cancer support organisations and government agencies it will provide:

• Identify key patient needs that can inform both therapeutic design and progress through the pipeline

• Provide thought leadership into the continually evolving policy landscape

• Foster greater collaboration to create breakthroughs in psycho-oncology and doctor-patient communication

• Identify potential gaps and opportunities to enhance patient safety and participation in clinical trials

Bring patient perspective to the fore

Patient advocacy groups may have a limited influence because they are based around a specific cancer type, hereditary syndromes or patient group. Their views and priorities may not be representative of a broader patient spectrum across our society.

To achieve greater impact, establish health based social network in Australia as an alternative to social listening because of its lower cost, greater accuracy patient choices, preferences and opinions as well as provide insight into adverse events.

A united patient advocacy organisation can give voice that truly matter to public opinion that would support and support regulatory bodies in their decision making with respect to the emerging treatments for cancer.

We need to bring out the voice of cancer patients, survivors and their caregivers and elevate the patient to be driving force behind innovation in healthcare. It is the united voice of the patient that can bring forward the promise of real-world evidence for a happier, healthier future for all of us in these uncertain times.

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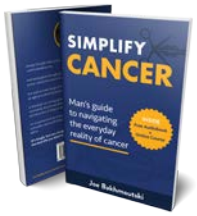
About

Joe Bakhmoutski

Founder, Simplify Cancer

Celebrated speaker, author, and coach Joe Bakhmoutski uses his lived experience with cancer and mental health struggles to share inspiration and advice on how to lead a happier, more fulfilled life after cancer. His Simplify Cancer Podcast featuring cancer experts and cancer survivors who share inspiration and advice for living a better life beyond cancer.

Praise for *Simplify Cancer: Man's Guide to Navigating the Everyday Reality of Cancer*



Foreword by Suzanne Chambers AO

Joe's Simplify Cancer podcast is an asset for anyone facing a cancer diagnosis. With this book, he's used his expertise to assemble a valuable guide for dealing with something that many have overlooked in the past – the psychological impact of cancer.

Dr. David Palma, MD, PhD, author of Taking Charge of Cancer

Hearing the three words "you have cancer" is devastating. Receiving this news is overwhelming. Joe has helped to make the experience a little less stressful by creating simple, easy to understand tools to help those affected by this disease navigate the complexities of Dr. Visits, treatments and so much more.

Lee Silverstein, We Have Cancer Podcast Host and Stage IV Survivor

Joe has used his personal experience to craft a simple, practical and meaningful strategy to help others in facing their cancer diagnosis and in taking back some control of their journey. Men facing a new diagnosis are sure to find Simply Cancer a great resource.

Mike Craycraft R.Ph., Survivor/Founder, Testicular Cancer Society

Dealing with cancer is tough and can leave people feeling scared and isolated. Joe's been through the wringer and has distilled what he's learnt from his cancer experience into this easily understandable and relatable book, Simplify Cancer. He offers personal and practical advice on everything from making an informed treatment decision, to getting support, and managing worries about the future. I hope this book will help those unlucky enough to be affected by cancer feel less alone and that they can live a personally meaningful life wth and beyond cancer.

Allan 'Ben' Smith, PhD, Centre for Oncology Education & Research Translation (CONCERT), Ingham Institute & UNSW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