Consumers Health Forum of Australia

SUBMISSION

House of Representatives Standing Committee on Health, Aged Care and Sport inquiry into approval processes for new drugs and novel medical technologies in Australia

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Submission to the House of Representatives Standing Committee on Health, Aged Care and Sport inquiry into approval processes for new drugs and novel medical technologies in Australia

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Introduction

The Consumers Health Forum of Australia (CHF) welcomes the opportunity to make a submission to this inquiry and thanks the members of the Standing Committee on Health, Aged Care and Sport for the opportunity.

CHF is the peak body representing consumers of health services in Australia and is Australia's leading advocate on consumer healthcare issues. We work to achieve safe, good quality, timely healthcare for all Australians, supported by the best health information and systems the country can afford.

CHF member organisations reach thousands of Australian health consumers across a wide range of health interests and health system experiences. CHF policy is developed through consultation with members, ensuring that CHF maintains a broad, representative, health consumer perspective.

CHF is committed to being an active advocate in the ongoing development of Australian health policy and practice.

Background

In recent decades there have been advances in technology, pharmaceuticals, more personalised medicine, and more targeted health deliveries. This has brought with it more complexity: greater costs, and greater expectations around quality healthcare mean substantial challenges in maintaining a viable and responsible medicines industry. It also means needing to restrict the use of some medicines to address challenges like antimicrobial resistance, which the World Health Organisation has stated as being one of the top 10 global public health threats facing humanity, driven largely by the misuse and overuse of antimicrobials and antibiotics, leading to the development of drug-resistant pathogens.

The increase in use and dependency on medicines, devices and diagnostics means we need to rethink how we remunerate and incentivise industry to develop new antimicrobials. Providing remuneration and incentives based on medicine sales, as per their unit price multiplied by volume of sales, is no longer a rational approach when medicines need to be used judiciously. It is potentially harming the health and the hip-pocket of consumers.

This inquiry sits firmly within the framework of the original four pillars on which the national medicines policy sits, namely:

- timely access to medicines that are affordable to individuals and the community
- high-quality medicines that are safe and effective
- the quality use of medicines
- maintenance of a viable and responsible medicines industry.

The healthcare environment has changed drastically since the national medicines policy was first implemented over 20 years ago. Change theory and community development principles

recognise that we need to identify the long-term goals and work backwards from there.[1] Identifying the conditions and contexts that have led to change can determine what conditions and outcomes need to be achieved for new goals to occur. A central principle of the policy is about keeping the consumer at the centre of a co-created experience. Because of this there is a growing expectation that consumers be informed and active in managing their own health and healthcare. Health providers also need to be better equipped to deal with people who are aware of the broader spectrum of treatments that are now available. Health providers need sufficient knowledge of proven therapies and their effective integration into the healthcare delivery system. Medication safety and efficacy therefore needs much greater surveillance. Training and credentials in the quality use of medicines—particularly in combination with other treatments—is more important than ever for primary healthcarers. The spectrum for health providers is possibly wider than it has ever been.

Changes in healthcare mean there is more room for gaps in care, which can lead to preventable harm. Consequently, more and better funding needs to be directed to those health services that provide non-pharmacological therapies and other treatments alongside drug treatment options. There is a set of National Indicators for the Quality Use of Medicines in Australian Hospitals, implemented in 2014. This includes continuity of care when patients are discharged, but there are currently no indicators specifically for community care. This separate set of indicators must be considered.

Jurisdictional differences are also making access to affordable and off-label medicine inequitable. Regional and remote health cannot be left behind when it comes to funding for community-based health. This includes access to quality aged care. Considering that Australia has the highest poverty rate in the OECD for aged pensioners, the inequalities around aged care have been a sad and awful highlight of COVID-19.

Support for member recommendations

Many of CHF's consumer-led member organisations have put in submissions, and some have appeared before the Committee. At the time of the submissions, this included the following organisations and CHF broadly supports their recommendations:

Fabry Australia
Migraine Australia
MS Australia
NeuroEndocrine Cancer Australia
Pain Australia
Rare Voices Australia

The overwhelming response to this inquiry is further evidence that the National Medicines Policy must be reviewed. As it stands, the policy clearly does not give guidance as to how to deal with novel treatments, new uses for existing medicines and other technologies, streamlining clinical trials and the incentives to research in these areas.

[1] Connell, J.P. and Kubisch, A.C., 1998. Applying a theory of change approach to the evaluation of comprehensive community initiatives: progress, prospects, and problems. *New approaches to evaluating community initiatives*, *2* (15-44), pp.1-16.

Terms of Reference

The following recommendations, which include feedback from health consumers who are members of CHF's safety and quality special interest group, are largely consistent with each other. They confirm that streamlining processes, working collaboratively with consumers and consumer organisations, embracing personalised medications, research and health technologies, supporting equitable access and the revision of the National Medicines Policy are all timely and necessary.

1 New drugs and novel medical technologies and the interface issues

The first term of reference looks at the range of new drugs and emerging novel medical technologies in development in Australia and globally, including areas of innovation where there is an interface between drugs and novel therapies. Developing a framework for the introduction of medications and medical technologies with clear guiding principles is vital.

The overarching goal of any review into new drugs or novel medical technologies is to improve the health of Australian people.

Framework governing principles

The framework needs to provide a comprehensive integrated system of healthcare with access to a full suite of care needs to optimise the use of medications and medical technologies, for example, through national clinical practice guidelines related to 'healthcare variations' being investigated and defined by the Australian Commission on Safety and Quality in Health Care. A centralised approach requires adequate resourcing but will increase transparency and accountability.

The framework must provide safe, timely, effective, person-centred/partnered healthcare within an equitable and sustainable healthcare system that is responsive to people's healthcare needs, values, and preferences. It must also include disease modifiers such as severity, equity, and uncertainty about the extent of treatment benefits in the real world for complete relevance and trustworthiness. Maintained within Australia, it would set the foundation for a responsible and viable medicines and medical technology industry with international credibility.

Transparency, fairness, accountability and democratic legitimacy

- Where 'democratic legitimacy' incorporates a broader view of evidence to inform efficacy and utility, effectiveness, and equity of allocation of resources, it can achieve a broad range of goals and outcomes.
- The goals can differ from the scientific paradigm, where 'scientific legitimacy' involves the application of scientific rigour and objectivity, leading to scientific policy goals rather than population goals.

Patient and public involvement

A purpose of patient involvement is to improve the legitimacy of decision making and is instrumental in producing better quality decisions that reflect patient and public preferences and values.[2] [3] [4]

Health Technology Assessment

Important developments in science and technology are leading to dramatic changes in healthcare and the need for Health Technology Assessments (HTAs). Examples include vaccines and digital technology. We are seeing new vaccines and medications and emerging novel medical technologies particularly in the areas of biologics, cell and gene (modified) therapies; tumour agnostic (histology and site independent) cancer therapies. Translational research is moving basic science discoveries more quickly and efficiently into practice, as we have seen with COVID-19 vaccines. Big data and precision medicine all have a role to play. We need to put in place the digital infrastructure to capture data on health outcomes in the short and longer term so that we can collect, use and learn from real world data and evidence.

HTAs can play a key role in providing equitable and sustainable universal healthcare. To ensure HTAs are conducted in the interest of patients we would like to see a clear and meaningful role for patients, carers and patient advocates in clinical assessments and appraisals along the entire HTA pathway, from scoping and prioritisation of HTAs to the development of recommendations and dissemination.

Standardising clinical assessments must be included. There are two key areas that enable access to new medicines and medical technologies in Australia:

- the process for registering a product for marketing in Australia (regulatory)
- the process to achieve government reimbursement (through PBAC, MSAC and the Prostheses List Advisory Committee).

Streamlined processes

To avoid waste of effort and duplication in regulatory and HTA processes, any framework must include the potential to create a single point for industry to engage with timely scientific advice, together with effective horizon-scanning. Working collaboratively with consumers and consumer organisations to access and understand real world data around co-design, disease-specific, patient relevant/patient-reported health outcomes' (PROMs) and patient-reported experience measures (PREMs), quality of life and patient preference data, must be included as part of the clinical trial, regulatory and health technology clinical assessments.

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[2] Boothe, K., 2021. (Re) defining legitimacy in Canadian drug assessment policy? Comparing ideas over time. *Health Economics, Policy and Law*, pp.1-16.
[3] Abelson, J., Giacomini, M., Lehoux, P. and Gauvin, F.P., 2007. Bringing 'the public'into health technology assessment and coverage policy decisions: from principles to practice. *Health policy*, *82*(1), pp.37-50.
[4] Abelson, J., Forest, P.G., Eyles, J., Casebeer, A., Martin, E. and Mackean, G., 2007. Examining the role of context in the implementation of a deliberative public participation experiment: Results from a Canadian comparative study. *Social Science & Medicine*, *64*(10), pp.2115-2128.

Overall, a new, streamlined HTA framework would help determine the true value of any medicine or technology from a patient perspective. Drug development processes need to be streamlined for targeted, personalised therapies, particularly for patients with rare diseases who stand to benefit most from a targeted approached. Streamlined decision-making will benefit complex technologies where medicine and device technologies are combined; it would provide clarity on benefits and risks to the patient, and overall benefit to society. It would address links between early diagnosis, treatment, monitoring and quality of care through care bundles that optimise treatment benefits of medical technologies, with patients working in partnership and at the centre of care.

Speeding up the assessment process for new medical technologies would address over-servicing, improve equitable access to new therapies, promote accessible, sustainable innovation in healthcare and collect the required data and expertise to—be evidence informed—to collaboratively define and rank/prioritise areas of need using mutually agreed criteria. Efficient and globally competitive access to new medicines, therapies and vaccines in Australia can continuously be improved through progressive improvements to Australia's regulatory and reimbursement processes.

The global network

The suggested framework would enhance Australia's evaluation and approval processes for new drugs and novel technologies by working collaboratively and being part of a global network.

Internationally, the need for the continual evolution of HTA methodologies including evaluation/clinical assessment that are timely, appropriate and robust has been recognised. Keeping up to date and adopting new methodologies and approaches, with sufficient flexibility, will balance benefits and risks to patients and the health system.

Connected information systems can be very helpful in capturing and analysing real-world data. For that picture to be comprehensive, the data-gathering must be systemic: data collection, collation, storage and analysis can all be used to inform health policy development to include web-based systems. Real world evidence includes electronic medical/health records, registries, patient-reported data inclusive of quality-of-life data, qualitative research, use of surrogate outcomes, deciding which outcomes are to be included in an assessment which needs patient and clinician input, costing, monitoring over time, and analysis of uncertainties. This will better inform the public and address expectations about regulation and HTA.

Australia's HTA system needs to value, and therefore deliver, the latest medicines and emerging technologies by applying flexibility and agility to ensure appropriate valuation, including from a patient and carer perspective. The current PBAC assessment of medicines, for example, inadequately considers the evaluation of social and economic impacts of a particular intervention, therefore producing gaps in the assessment process. Economic evaluation of an intervention must be conducted within a societal perspective and broader context in mind.

Evaluation and improvement

Significant gaps exist for evaluation and improvement in the contribution and participation of patients and the public in HTA (from scoping to economic modelling, assessment, reporting and dissemination). These gaps apply to individual treatments and to the health system more broadly.

HTAs have increased in scale and complexity making them less accessible for patient groups. Summaries of HTA deliberations should be timely and accessible to inform the general population. Improved, streamlined pricing negotiation processes are also needed to enable greater transparency of funding arrangements across the health system. Patient groups need to be provided with the same timely information on the new interventions as the medical profession to enable them to meaningfully contribute to regulatory and HTA processes.

Methods are needed to incorporate data and evidence provided by patients into the assessment processes. Patients/consumers and carers provide essential contextual knowledge which they gain from living with a condition and using treatment interventions.

HTA systems need the mechanisms to fully recognise, and value the unique information patients have about a condition, its impacts, and the social value of treatments. Patients can also contribute an understanding of the importance of efficient and accurate diagnosis, integrated care, and the optimal use of treatment interventions.

Major challenges exist regarding the capacity and capability of patient groups to participate. This is in addition to working closely and sensitively with the patient-clinician community to identify and address unmet needs.

2 Incentives needed to research, develop and commercialise new drugs and novel medical technologies

The second term of reference looks at what incentives are needed to research, develop and commercialise new drugs and novel medical technologies for conditions where there is an unmet need, in particular orphan, personalised and off-patent drugs that could be repurposed and used to treat new conditions:

As well as new and novel, we need to look at new uses and novel uses for existing medicines and other technologies. Development of vaccines and treatments for COVID-19 and 'Long Covid', for example, have been based on novel uses for existing medicines and other technologies. Repurposing existing therapies may allow patients faster access to medicines.

Elevate the inclusion of repurposing existing medicines into novel treatment research funding options. The current preconception that profit is necessary to make research into repurposing medicines fruitful is restricting what makes it into the drug development pipeline.

Increase funding for research into novel treatment options particularly for rare diseases, which are often not suited to standard double-blind placebo-controlled research.

3 Measures to increase clinical trials

The third term of reference looks at measures that could make Australia a more attractive location for clinical trials for new drugs and novel medical technologies. An important part of research and development for new medicines and medical technologies is clinical research trials. By being involved in clinical trials, Australians (including patient advocates and clinicians) can have a greater say in their design including who is involved, for how long, and which outcomes are measured.

The uncertainty around funding mechanisms in Australia means there is reluctance with pharmaceutical companies to invest in and conduct clinical trials, particularly for rare diseases. Incentives for pharmaceutical companies must be strengthened to include expedited HTA approval processes and streamlined ethics processes.

Clinical trials build the expertise and preparedness of our health system in working with, and improving new, innovative technologies. Measures for clinical trials could include:

- Engaging consumers and consumer peak bodies by the Pharmaceutical Benefits Advisory Committee in approval processes, to ensure independent input is provided to inform deliberations
- Developing a national standard approach, in consultation with industry, including nationally agreed systems and standard operating procedures to support and strengthen the capacity to conduct clinical tele-trials in rural, remote, and regional areas, and including regional tele-trials in community awareness campaigns
- Co-ordinating national infrastructure to support clinical trials including streamlined, single-point ethics approval processes, tax incentives for businesses and additional investments in research and development, clinical trials, and commercialisation.
- A national approach to clinical trials via using multi-trial sites, sharing common resources and opportunities for patients in rural and remote areas to participate.

While insisting on consumer co-design, the low levels of health literacy in Australia mean clinical trials are often not understood by consumers. Strengthening health literacy must continue to be supported for increased consumer input.

4 Refinements to the approval processes

Medicines, medical devices and novel technologies are all part of a global market. This term of reference looks for ideas that would not compromise the assessment of safety, quality, efficacy or cost-effectiveness, but might make the approval process for new drugs and novel medical technologies, more efficient, including through greater use of international approval processes, greater alignment of registration and reimbursement processes or post market assessment:

In our submission into the 2015 *Expert Review of Medicines and Medical Devices Regulation* CHF argued that there should be greater use of assessments from other international regulators, and we still believe that this needs to happen. This is despite the significant improvements in medicine assessment, particularly the introduction of the provisional

approval stream which allows medicines to effectively be trialled in the real world once safety has been established.

Not all therapies meet the criteria for subsidy under the current pharmaceutical benefits advisory committee pathways. The Life Saving Drugs Program has provided specific treatments, but the inequity of access needs urgent review to be in line with international guidelines and standards. Reimbursement pathways are also still unclear and inequitable, particularly for rare disease treatments.

Every Australian, including those in rural and remote communities, should have access to clinical trials. Much has been done to improve patient's knowledge about clinical trials but there is still more to be done in terms of matching patients to trials and improving clinicians' access to up-to-date information on relevant trials.

One of the most difficult aspects of research is finding the right participants. Consideration needs to be given to providing financial support to people on trials, particularly those from rural areas who may have to travel many times or live away from home for the duration of the trial. In discussions with consumers about clinical trials this financial barrier is often raised as a reason for dropping out of a trial or not participating. Several key initiatives are occurring and must be considered as part of the considerations for future developments in this area:

- We strongly support the George Institute for Global Health's *Join Us* register. The *Join Us* register is an easy way for millions of Australians to participate in life-saving health and medical research. It is a secure register that matches participants with research studies addressing Australia's biggest health challenges
- The Australia Commission on Safety and Quality in Health Care is currently conducting national consultations on behalf of all jurisdictions to scope the requirements for a national health and medical research approvals platform—a national One-Stop-Shop—aimed at improving and streamlining research pathways for patients, researchers, industry representatives and sponsors
- The US passed federal right-to-try laws in 2018, allowing thousands of terminally ill patients to access therapies that have completed Phase 1 testing but have not yet been approved the US Food and Drug Administration. CHF has not formally canvassed consumer views on right-to-try arrangements. While there are clearly many problems with such arrangements, not least of which is getting informed consent, we think it is time to have a more open conversation about this option and its risks and merits. Consumers must be part of the sponsor registration process, including intent to seek reimbursement in Australia.

Conclusion

These recommendations confirm that streamlining processes, working collaboratively with consumers and consumer organisations, embracing personalised medications, research and health technologies, embracing developments in science and technology and supporting equitable access requires the foundational policies around medicines and the medicines industry be reviewed.

With all this in mind, CHF's goal is to shape better healthcare by ensuring that the primacy of the consumer remains front and centre of policy development. This development must include a clear plan and commitment to fully implement new guidelines and systems for there to be any real change and improvement to healthcare in Australia. New drugs, therapies, challenges, and opportunities are being introduced every day to healthcare.

The national medicines policy and, importantly, associated implementation must be updated so that it can be adaptive to the many new medicines, therapies, challenges, and opportunities that present themselves every day in the health sector.

Data measuring and evaluation must be prioritised for real world impact to continue and so that appropriate adaptations can take place. This will also continue to strengthen and focus funding decisions in the dynamic and ever-changing world of healthcare. The ultimate results will be better data, better health outcomes, better partnerships, and a better future for all.

Governments need to work together to set out short, medium, and long-term agendas for action. The simpler and clearer the visions and principles of the National Medicines Policy can be, the better it will help health consumers and the health sector understand what world class should look like. The agendas need to be clear about the obligations placed on each Commonwealth department and each state and territory government. They need to give consumers confidence that they can get the care they need, when they need it, and they need assurances that the system is properly funded and sustainable.