NATIONAL MEDCINES POLICY REVIEW

National Policy Review Committee

October 2021

Genetic Undiagnosed And Rare Disease (GUARD) Collaborative Australia

GUARD Collaborative Australia comprises peak body organisations; Genetic Support Network of Victoria, Genetic Alliance Australia (NSW), Syndromes Without A Name (SWAN) Australia. Together, we represent the voice of people living with genetic, undiagnosed and rare disease and those who support them. We strive for a fair, equitable and collaborative approach to health, disability, education and the wellbeing for all our community.

GUARD members work independently of each other fulfilling their own responsibilities and roles and come together to provide systemic national advocacy and support for people living with genetic, undiagnosed and rare conditions and those who support them. Our advocacy is driven by values of fairness, equity and quality of life and focusses on change in the health and disability sectors.

Together we offer our united collaborative strength and provide assistance, support and services for genetic, undiagnosed and rare condition support groups, their members and the wider community seeking to influence and change current health, mental health, support services and disability policy and practice impacting our community. We strive for a fair, equitable and collaborative approach to health and wellbeing for all.

The work of GUARD is consistent with the National Strategic Action Plan for Rare Diseases, national and state genomics and precision health policies and our individual organisation objectives.

This submission was prepared by GUARD Collaborative Australia in consultation with our Community Advisory Group and broader genetic, undiagnosed and rare disease community.

GUARD Collaborative Australia

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GUARD Collaborative Australia acknowledges the Traditional Custodians of the land and pay our respects to their Elders past, present and emerging.

guard.org.au

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Executive Summary

GUARD welcomes the review of the National Medicines Policy Review which will ensure it remains fit for purpose with future advances in medicines and healthcare. We have addressed all six terms of reference in our submission as they are relevant to the communities we support.

In Australia, it is estimated that 60% of the population will be affected by a condition with a identified rare conditions rises daily, it iw estimated there are approximately 8000 rare diseases. These statistics are expected to increase. Recently, the Australian Bureau of Statistics reported n the number of people with rare disease is equal to diabetes.

The diagnosis of a genetic condition presents challenges and may ignites deep and intimate beliefs and questions about ourselves, those who came before us and those who come after. The review of the National Medicines Policy and its implementation will have a significant impact on the lives of people living with rare disease.

The organisations listed below have contributed and support the comments for consideration in the Review of the National Medicines Policy.

ADD LOGOS HERE

Summary of Key Issues and Recommendations

KEY ISSUES	RECOMMENDATIONS
1/ Proposed Principles & Objectives	**<u>Suggested amendments to the proposed principles:</u>** **Consumer Centred Approach** ● Consumers should be informed, engaged, and empowered through inclusive and transparent processes to participate in medicines policy, recognising their key role in supporting the achievement of the policy's objectives. **Accountability and transparency** ● All stakeholders are identified and accountable for their responsibilities and actions towards delivering or contributing to the achievement of the policy's objectives, within a transparent framework that engenders trust and demonstrates an ongoing commitment to collaborative outcomes of policy objectives. **Innovation** ● All stakeholders have a shared responsibility to ensure investment and benefit for the diverse community of Australia in development, re-use and re-purposing, exploration, discovery and translation of health treatments and therapies. **Regulatory Practice and Compliance** ● Inclusion of compliance principles with transparent evaluation to ensure the highest level of standards, communication, regulations and statutory obligations are always adhered to.
2/ Definition of Medicine	● The single or simultaneous use of pharmacological, chemical, immunological, genetic, mechanical or metabolic regimes in or on the human body.
3/ New Policy Title	• National Health Treatments and Therapies Policy
4/ Changing Environment	**The policy needs to take into consideration:** ● Responsiveness ● Process ● Equity of Access ● Lived Experience

5/ Centricity of Consumers	**The policy needs to take into consideration:** ● Consumer engagement shifts from passive to active involvement ● Lived Experience ● Inclusion of people from CALD backgrounds, First Nations people and other diverse backgrounds such as disability, ageing, LGBTQI and diverse disease categories including rare. ● Dynamic Change
6/ Governance, Communication and Implementation	**The policy needs to take into consideration:** ● Stakeholder engagement ● Consumer as part of the governance model ● Dynamic Change ● Delivery ● Governance ● Improved communications – timely, accurate, trustworthy and transparency

Introduction

Thank you for the opportunity to provide feedback to the National Medicines Policy Review Committee on the principals and objectives of this policy. It is important that we express the views of GUARD, our community advisory group and broader genetic, undiagnosed and rare disease community in this submission.

The National Medicines Policy shapes Australia's approach to the use of health treatments and therapies. Partnerships with key stakeholders, including that of consumers is pivotal in ensuring all Australians have equitable access, the opportunity to benefit from advances in technologies and knowledge and timely access to quality, safe and effective medicines.

We commend the government's ongoing commitment and approach to broad consultation and collaboration, ensuring our community has the best opportunity to stay healthy and well.

Terms of Reference

1. Evaluate the current NMP objectives and determine whether these should be modified or additional objectives included. This includes consideration of the proposed Principles to be included within the NMP.

RECOMMENDATIONS:

A suggested amendment to the proposed principles and two additional principles:

Consumer Centred Approach

Consumers should be informed, engaged, and empowered through inclusive and transparent process to participate in medicines policy, recognising their key role in supporting the achievement of the policy's objectives.

Accountability and transparency

All stakeholders are identified and accountable for their responsibilities and actions towards delivering or contributing to the achievement of the policy's objectives, within a transparent framework that engenders trust and demonstrates an ongoing commitment to collaborative outcomes of policy objectives.

Innovation

All stakeholders have a shared responsibility to ensure investment and benefit for the diverse community of Australia in development, re-use and re-purposing, exploration and discovery.

Regulatory Practice and Compliance

Inclusion of compliance principles with transparent evaluation to ensure the highest level of standards, regulations and statutory obligations are always adhered to.

a) a) Are these proposed principles appropriate? With regard to the proposed principles, is anything missing or needing to change?

We support and believe the proposed principles of; equity, consumer centred approach, partnership based, accountability and transparency and stewardship as appropriate. These proposed principals need to inform every stage of planning, design and implementation of programs and initiatives delivering this policy and ensure the policy is fit for purpose and will continue to remain so until it's next review. We have addressed the proposed principals in more depth below:

Equity

The principle of equity goes beyond provision of effective, safe, high-quality and affordable medicines. Barriers, creating inequities in access to medicines do not just arise from people's social, economic or cultural background, or from social determinants of health. They can also arise from lack of investment in research, medicines and treatment plans due to disease rarity or have unknown origin. True equity will require a commitment in this policy to support innovation, discovery and translation. Innovation has been recommended as an additional principle.

Consumer Centred Approach

We welcome the inclusion of the principle of a consumer centred approach. While we agree that consumers should be informed, engaged, and empowered, we strongly suggest that this principle be enhanced. Importantly, consumers should be involved in every stage of the planning, design, implementation, and evaluation of programs, systems and initiatives. Consumers play a key role in and reshaping the development of strategies that achieve the policy's objectives. Consumers should represent a diverse range of stakeholders from diverse disease conditions (including genetic, undiagnosed and rare disease communities), disability, ageing, LGBTQIA+ and cultural communities.

Lived experience should be prioritised along with people from the communities which support them. Consumers who represent communities should also be supported to engage in committees through what eve means they need support, e.g. communication and/or accessibility. This will ensure they can contribute to committees in a valuable way.

A suggested amendment of the principle is:

> *Consumer centred approach – consumers should be informed, engaged, and empowered through inclusive and transparent process to participate in medicines policy, recognising their key role in supporting the achievement of the policy's objectives.*

A suggested amendment of the principle is:

> *Consumer centred approach – consumers should be informed, engaged, and empowered through inclusive and transparent process to participate in medicines policy, recognising their key role in supporting the achievement of the policy's objectives.*

Partnership based

GUARD believes that the principle of partnership should be more than establishing/maintaining partnerships and harnessing each other's strengths. The implementation of this principle should overtly demonstrate trust, genuineness, and commitment. This has been included in the accountability and transparency principle. Emphasis should also be placed on the commitment to collaborate with consumers and consumer groups as true partners in the process of achieving the NMP's objectives.

Accountability and transparency

Stakeholder engagement and consultation needs to be complemented by stated actions that enable the community and internal processes to evaluate this principle. All decisions made by stakeholders based on consultation need to be overt, transparent and evaluated so there is accountability for actions taken.

A suggested amendment of the principle is:

> *Accountability and transparency – all stakeholders are identified and accountable for their responsibilities and actions towards delivering or contributing to the achievement of the policy's objectives, within a transparent framework that engenders trust and demonstrates an ongoing commitment to collaborative outcomes of policy objectives.*

Stewardship

Stakeholders have a shared responsibility to ensure benefit, equitable access, cost containment, ethical application and efficiency for the Australian community. As stewards of the health system, all stakeholders also have a responsibility to ensure quality of healthcare. Stewardship needs leadership, accountability etc. from the government itself a single driver to make sure the policy is properly implemented and nothing and nobody falls between the gaps – leadership is provided to steward the policy and ensure that all stakeholders are included, incorporated and communicated with for effective implementation

Not only should these principles be evidenced in the planning, design and implementation of programs, systems and initiatives, they should form the bases of the evaluation of such programs, systems and initiatives.

A healthy community has value beyond financial, adding to the social capital of the community. A healthy community adds to intergenerational cohesiveness, strengthening bonds and community participation in social activities as well as employment opportunities. This must not be forgotten by the implementers of the policy.

Additional recommendations to the NMP Principles:

Innovation

With the increase in genetic technology, a corresponding increase in identification of rare genetic conditions is expected. Stakeholders must commit to innovation to ensure equity of access to health treatments and therapies and subsequently quality of life. This is consistent with the Department of Health, National Strategic Action Plan for Rare Diseases.

By their nature rare conditions are difficult to research. However, recent advances in genomic technologies provide more opportunity and more hope. The development of treatments and therapies remains time consuming, if possible. Delays can mean lost lives or diminishing quality of life and future treatment options. Pharmaceutical companies may choose not to develop treatments and therapies or not to apply for regulatory approval in Australia due to the commercial challenges of market size and potential returns in genetic and rare disease products. The current processes for approval or subsidized access may also act as a deterrent. The NMP must encourage and support innovation as an enabler to equity.

Suggested wording for inclusion of this principle is:

> *Innovation –All stakeholders have a shared responsibility to ensure investment and benefit for the diverse community of Australia in development, re-use and re-purposing, exploration, discovery and translation of health treatments and therapies*

Regulatory Practice and Compliance

The NMP must include Regulatory Practice and Compliance as an underpinning principle. These are the mechanisms which ensure the protection of consumers in the delivery of the policy. Regulatory practice and compliance must enshrine that health consumers have informed choice of health intervention through a decision making framework accessible to all in every case. It is only through transparent, consistent application of the policy and all policy principles, evidenced through evaluation that the public trust is built and maintained.

Suggested wording for inclusion of this principle is:

> *Regulatory Practice and Compliance – Inclusion of compliance principles to ensure the highest level of standards, communication, regulations and statutory obligations are always adhered to.*

b) Are these four Objectives still relevant? Should any be modified, or any additional objectives be considered? If so, how and why?

The objectives of the NMP are still relevant and must be clearly linked to the principles. Factors to be considered are included below:

Access to medicines

Access to medicines is not equitable for all. Many factors influence access for those with genetic, undiagnosed and rare conditions. For most, there is no treatment or therapies available at all or if they are, they are not approved in Australia. If they are approved they may be financially unattainable.

For some, the only hope of access to treatments is through clinical trials. While clinical trials reduce treatment cost, access is often restricted to those in metropolitan regions. Regional, rural and remote residing individuals may not be able to fund the out-of-pocket expenses associated with travel and accommodation to attend trial appointments. At completion of a trial continued access to treatments is not assured and may be reliant on industry compassionate access and commercial

interests to apply for access under the Pharmaceutical Benefits Scheme (PBS). Research and trials must be recognized as an issue of equity in the National Medicines Policy and its implementation.

Access to medicines is a complex objective which must be achieved as a universal human right in Australia.

Quality, safety and efficacy of medicines

The reference provided for quality, safety and efficacy of medicines references 'comparable countries' but does not list which countries are included. As the Policy was written 20 years ago, it is totally possible that the referenced 'comparable countries' have changed their policies and internal processes or changed their health landscape and subsequent needs. These must be viewed as not mutually exclusive with one impacting the other as it occurs. An alternate reference benchmark to gold standard universal health systems may be more suitable and fluid, which in turn will assist in future proofing the Policy.

Quality use of medicines

The quality use of medicines, treatments and therapies is central to the National Medicines Policy- to reduce preventable harm and the promotion of optimal health outcomes. The contemporary maxim of 'the right treatment, at the right time' applies here. The quality use of medicine also requires that the right patients are *identified* at the right time - access and quality use are dependent on timely diagnosis. Diagnostic pathways such as Newborn Screening, reproductive carrier screening and the use of genomic technologies would greatly strengthen the implementation of this objective. The NMP must include precision medicine implementation as part of the objective of quality use. For for rare and undiagnosed conditions, the 'right' treatment' may be unknown, unresearched and therefore unavailable. Or available as incredibly expensive options for those with choices. Diagnostics, medicines and treatments may be repurposed from more common conditions potentially providing a little relief, a band-aid to symptoms, and are generally dependent on advocacy by the patient or parent for better treatments. The concern of polypharmacy and adverse outcomes is more difficult to predict in rare and undiagnosed conditions, resulting in additional consultations and costs with financial, time and social impact on families.

The quality use of medicines is of real significance for those with rare conditions through the provision of the Life Saving Drugs Program. Provision of medical intervention that enables life is viewed universally as compassionate and invaluable. Unequivocally, provision and access to medicines, treatments and therapies is a human right. For this reason, life-saving drugs need to be included in the quality use of medicines as necessary under Quality, Safety and Efficacy of medicines.

Post market reviews need to include Patient Reported Outcomes and Patient Reported Outcome Measures, not only quality of life measures. PROs and PROMs matter for those with genetic, undiagnosed and rare conditions. Whether in the home, health service delivery and hospitals the opportunity for post market review needs to encompass a variety of locales, social expectations, and delivery effectiveness. Post market reviews also need to include medical misadventure, over the counter medicines, complementary medicines and evaluate how data can be more effectively and efficiently gathered to ensure just-in-time review, quality improvement, efficacy and potential positive/ negative interactions with prescribed medications and optimal patient outcomes.

Further, the NPS must recognize alternate and traditional medicines (utilised by first nations peoples for use by their community) and the potential benefit for the wider Australian community.

The public as the ultimate end user of medicines and therapies, must be engage to inform efficacy and effectiveness.

Maintaining a responsible and viable medicines industry

To stimulate and maintain a viable, responsible and sustained medicines industry, the NMP implementation must encourage a broad, cohesive, transparent and collaborative approach by all stakeholders including consumers, industry and government. A viable medicines industry includes supporting pre-clinical and clinical research, especially in areas such of low commercial interest

(repurposing, rare disease) - therefore the NMP needs to be explicit about appropriate support & investment in R&D and clinical trials so that the potential benefits of new medicines can be realised.

The challenge of the COVID-19 pandemic has thrown focus on the supply chain for medicines as well as other needed clinical materials from a viable medicines industry. A GUARD Collaborative Australia survey in 2020 to those with genetic, undiagnosed and rare conditions showed 59% of our community experienced difficulty having a pharmacy prescription filled. This must be addressed and may require the review of criteria for repurposing of medicines which has greater urgency in increasingly challenging current and future health environment

It is also critical that this policy is developed to support the 10 year and beyond horizon and health targets in other key policy documents. They cannot be developed and implemented in isolation.

2. Consider the definition of medicines and whether the NMP needs to be expanded to include health technologies

RECOMMENDATIONS:

A suggested definition of medicine is:

The single or simultaneous use of pharmacological, chemical, immunological, genetic, mechanical or metabolic regimes in or on the human body.

A suggested title for the National Medicines Policy:

National Health Treatments and Therapies Policy

a) Should the current NMP definition of medicines be expanded to include medical devices and vaccines? Why and Why not? How would a change in definition of medicines be reflected in the policy's high-level framework

Definitions of Medicine:

Dictionary definition:

- The science or practice of the diagnosis, treatment, and prevention of disease (in technical use often taken to exclude surgery).
- a drug or other preparation for the treatment or prevention of disease.

Therapeutic goods[1] generally fall under three main categories:

- Medicines - including prescription, over-the-counter and complementary medicines, such as paracetamol and echinacea
- Biologicals - something made from or containing human cells or tissues, such as human stem cells or skin
- Medical devices - including instruments, implants and appliances, such as pacemakers and sterile bandages Note: this would include Software as a Medical Device (SaMD)

The definition of medicine to include 'prescription and non-prescription medicines' omits the inclusion of contemporary technologies to treat illness and chronic conditions.

[1] https://www.tga.gov.au/what-are-therapeutic-goods

For genetic, undiagnosed and rare conditions, a 'cure' may not be an achievable end goal. Currently, for most of these conditions, health and quality of life is dependent on lifelong management of symptoms, sometimes including family planning so as not to pass inherited conditions to offspring or to delay progression of disease.

Further, multimodal therapies including devices (for example: glucose measuring and insulin dispensing monitors, cochlear implants), are becoming standard of care in genetic, undiagnosed and rare conditions. To be more inclusive and to holistically consider all aspects of the management of conditions, the term 'medicines' to be changed to 'treatments and therapies' or 'medical intervention'. A new definition needs to reflect the health technologies of medicine, genomics, devices, prosthetics and surgery procedures.

A suggested definition of medicine is:

'the single or simultaneous use of pharmacological, chemical, immunological, genomic, mechanical or metabolic regimes in or on the human body'.

b) Does the policy's current title, the National Medicines Policy, reflect the breadth of health technology developments with the policy's scope? IF not, how best can these and future health technologies be better represented in the policy's title

The current title 'National Medicines Policy' has served well for 20 years, but now needs to reflect contemporary treatments and therapies.

A suggested title is:

'National Health Treatments and Therapies Policy' or 'National Therapeutics Policy'

By including therapies and devices, this enables newer technologies and those that are developed in the future, to immediately fall within the NPS. This is also consistent with changing process within Health Technology Assessments incorporating new interventions for improved health.

3. Assess the NMP's utility in the context of rapidly evolving treatments options, population changes, interconnected relationships and system-wide capacities

RECOMMENDATIONS:

Ensure the following are considered when modifying the NMP:

- Responsiveness
- Process
- Equity of Access
- Lived Experience

a) How has the NMP been able to maintain its relevance and respond to the changes in the health landscape?

The NMP has been able to accommodate extraordinary situations as evidenced by the COVID-19 pandemic. As technologies and consumer health literacy bring new possibilities, ethical challenges and informed consumers, our NMP must reflect this new environment. Our community expects the NMP will include the following areas:

Precision medicine

The increasing use of gene analysis technology has enabled diagnosis and treatments not previously possible. However, many conditions remain undiagnosed as this technology and knowledge is in the early discovery phase. Also, for common conditions there are anomalies that do not respond to current medicines and treatments. These outliers, in some cases, are being shown to have variations in gene expression. Personalised therapies are required to deliver the promise and hope of a quality life for individuals. We have the technologies. For this reason, the revised National Medicines policy will need to be future proofed to encompass this aspect and breadth of treatments.

Health literacy

Parents and individuals with genetic, undiagnosed and rare conditions become knowledgeable about their conditions, educating clinicians and other health and service professionals, becoming the expert. These experts become more vocal, they share their experience with Patient Support Groups and condition communities broadening knowledge and demand of and for treatments. This also contributes to understanding of efficacy on a broad level. This informal knowledge sharing, empowers consumers and may reduce unsuitable treatments and time to improved health.

However, those in CALD communities generally experience dual isolation due to language and the rarity of the condition. Where, health literacy is reduced, vulnerable populations such as CALD, indigenous, LGBTQI, non-verbal communicating and others living with genetic, undiagnosed and rare conditions may be further marginalised and less empowered in health decision making. Building health literacy and informed decision making for all must be responsibility examined as part of the implementation of the NMP.

Regulatory controls

Regulatory bodies such as the TGA and PBAC must ensure that under a new definition of 'medicines' that there is appropriate and timely process development to ensure clarity, equity, transparency and efficiency to progress access to treatments and therapies in this country. These may be complementary or included in current processes, but not add complexity and confusion for stakeholders and authorities.

Equity and sustainability

Treatment for rare disease has been driven often by passionate individuals, motivated to make a difference for those affected today and into the future. The need to ensure greater equity, followed by sustainability is a cornerstone to support the needs of the community into the future. Investment into pioneer treatments and discovery for genetic, undiagnosed and rare conditions requires active support from those with stewardship responsibilities, notably industry and government.

Real -world evidence

The use of PRO, PROMs, electronic devices, registries, big data and AI can contribute to real world evidence. These have had limited application in Australia compared to other countries. Policy to enable the collection of this data needs to consider confidentiality, ethical use, consent to participate, social bias and discrimination, as well as address legal and employment implications.

Issues of data access, privacy, and ownership are always raised as barriers to effective use of real world evidence. There are numerous global (including Australian-led) examples of where these challenges have been overcome. The NMP could be strengthened to provide leadership and guidance to overcome this issue and drive real change in the national infrastructure that will make this possible.

Drug repurposing

Drug repurposing requires comprehensive review to encourage and facilitate active exploration. Additionally, with increasing genomic technologies and identification of like genetic signatures, medicines may be effective for several conditions. Current single condition purpose drugs may be more viable commercially if usage can be responsibly broadened under the principles and objective of the NMP. Exploration into drug repurposing needs to be made more accessible for appropriate stakeholders to enable treatment that allows testing possibilities on many levels including by organ or symptom, by genomic profile. The NMP could be stronger driving the change that is needed systemically to support the rapid testing and access to trials of repurposed drugs where there is low commercial interest e.g. systems and investment that support 'public good clinical trials'.

Digital health, telehealth and teletrials

While not encapsulated in the existing NMP, it is important to consider that the capacity and appetite for telehealth and teletrials has increased, stimulated by the pandemic and social distancing requirements.

Telehealth and teletrials enable education about and access to medicines and treatments, with less restrictions due to location-metropolitan, regional, rural, remote, albeit subject to internet access. For genetic, undiagnosed and rare conditions, this enables equity of access to specialists with knowledge of medicines and treatments. It is important to ensure technology discrimination does not occur and to recognize that access to computer technology and computer skills is needed to gain access to health services and subsequent medicines.

Communication needs to be in a medium that suits the user, barriers such as age and ability must be considered in an equitable system. Services such as e-prescribing need to be accessible by all Australians to ensure unintended discrimination and inequity.

b) How could the NMP be refreshed so that the policy framework is able to better address current and future changes in the health landscape? What is missing and what needs to be added to the policy framework and why?

To stay relevant and support current and future health environments, the NMP Policy Framework must consider:

Improved Responsiveness

Access to medicines is difficult to define especially for rare and undiagnosed conditions. Diagnostic and/or treatment delays can lead to devastating outcomes, including death, a different life course, a reduced quality of life and more. Adequate funding for evaluation and implementation of timely diagnostic opportunity must be provided to optimize the quality use of treatments and therapies. The diagnostic odyssey for rare conditions causes delays in accessing effective medicines, in which can lead to the use of unsuitable medicines. There are serious consequences that result from these delays. Including health as well as financial burden due to ongoing health concerns or unaffordable life savings medicines. Th NMP needs to be supportive and not contribute to problems and for these reasons, timely must be clarified, defined and be responsive.

Improved process

Processes supporting the implementation of the NMP policy such as the TGA, MSAC or PBAC systems of approval must support timely access to medicines, treatments and therapies. Adequate funding must be available to support the equitable implementation of the NMP through these mechanisms. These systems, funding and system guidelines must be regularly reviewed to maintain alignment with the NMP.

Improved equitable access

There are many benefits arising from advancements in genomic testing and precision medicine, however, equity across conditions does not exist Regular transparent evaluation of the NMP implementation on the public record must include support for discovery research and equity status for all Australian consumers

Evaluation of the NMP implementation against the principles including partnering with stakeholders to ensure equitable access is a value subscribed to by all will ensure the NMP continues to be accountable and responsive to changing demands.

Lived experience

The recent discussion on the use of research and changes to HTA to include lived experience is very welcome. We stress the importance of ensuring that lived experience includes groups such as the regional, rural and remote communities, diverse communities including LGBTQI, genetic, undiagnosed and rare-disease community, Non-verbal communicators, new mothers, First Nations peoples, and Culturally and Linguistically Diverse populations.

It is critical that the shared lived experience becomes part of the library of knowledge informing future direction for the NMP and instruments of implementation. It must be a living library accessed regularly, analysed for learnings and a benchmark for positive changes.

4. Consider the centricity of the consumer within the NMP and whether it captures the diversity of consumers' needs and expectations.

RECOMMENDATIONS:

Ensure the following are considered when modifying the NMP:

- Consumer engagement shifts from passive to active involvement
- Lived Experience
- Inclusion of people from CALD backgrounds and First Nations people and other diverse backgrounds such as disability.

a) How can the NMP's focus on consumer centricity and engagement be strengthened? Is anything missing and what needs to change?

Active consumer engagement

Current NMP states that "this Policy recognises the fundamental role consumers have in reaching these objectives, and there needs to be a commitment from all partners to ensuring consultation with consumer representatives when new arrangements are contemplated." To achieve the commitment of consumer-centricity there is a need to shift the role of consumer from passive engagement to active involvement as outlined in this responses below:

- Embed consumers at each level, inclusive at governance level
- Ensure cross section of consumers
- Consumers as co-chair
- Succession planning/training for consumers

Lived experience

To be truly consumer-centric, consumers should have a key role in contributing to all decision-making process of investments in medicines, treatments and therapies. Representation should be from those with lived experience or active engagement in the patient support community. A dedicated consumer committee comprised of these representatives should be enabled to meet, share knowledge, provide their expertise and provide peer support to other consumers involved in the processes of planning, design, implementation, and evaluation of programs, systems and initiatives. The policy can ensure consumers are well supported in the role on commitees.

The involvement of the consumer voice is greater than a seat at the table. The NMP is to serve the community, it is only fitting that the community is present and participating in decisions affecting the whole. Individuals may lack the energy and capacity to advocate on issues relating to health, there must be trust in representation and it must be for all. Training should be provided if required to ensure the consumer is confident in their role.

Diverse backgrounds and demographics

The inclusion of CALD and First Nations peoples is required in consumer consultations. Other vulnerable populations such as people living with disabilities, non-verbal communicators, LGBTQIA+, rural and remote communities and people living with genetic, undiagnosed and rare conditions must also be included. It is noted that "Medical products are safer and more effective for everyone when clinical research includes diverse populations."[2]

There are currently no requirements in Australia for sponsors to report on demographic subgroup data. Little is known about the rate of subgroup enrollment in Australian clinical trials, and there is currently no publicly available demographic subgroup data on medicines in Australia. Those in regional, rural and remote localities need to be included, assisted by telehealth and teletrials for genetic, undiagnosed and rare conditions.

In the United States, section 907 of the Food and Drug Administration Safety and Innovation Act (FDASIA) requires the FDA to investigate how well demographic subgroups (sex, age, race and ethnicity) in applications for medicines and medical devices, submitted for marketing approval, are included in clinical trials, and if subgroup-specific safety and effectiveness data are available. Australia must learn from this and implement such granularity to ensure equity.

Dynamic change

The reviewed NMP will serve the Australian population during a time of dynamic change. Genomics is already changing the face of mainstream heath delivery, challenging possibilities, changing trajectories, creating new pathways for treatments and therapies and driving precision and preventative health and wellbeing.

The consumer-centricity for NMP must also be dynamic. As the options and possibilities change for health consumers the NMP must stay ahead, creating policy certainty for regulators, agencies, industry, government, health professionals and consumers.

The living guidelines model should be applied to create a living policy that retains utility and relevance through changing times. The living guidelines model feeds global research findings, developments and lived experience into a dynamic model to review current practice and inform potential change to health delivery, treatment and therapies. Clinical expertise is used to assess the utility of the research or knowledge. A consumer panel (with diverse representation) would partner to form part of the living guidelines governance model and inform potential implementation challenges for the policy evolution and unintended consequences for the consumer community.

[2] *https://www.fda.gov/regulatory-information/food-and-drug-administration-safety-and-innovation-act-fdasia/fdasia-section-907-inclusion-demographic-subgroups-clinical-trials*

Australia successfully adopted this inclusive and progressive model for the development and implementation of clinical guidelines during the COVID-19 pandemic and utilizes this living guidelines model for the treatment development in some specialist areas.

5. Identify options to improve the NMP's governance; communication, implementation (including enablers) and evaluation.

RECOMMENDATIONS:

Ensure the following are considered when modifying the NMP:

Stakeholder engagement

Consumer as part of the governance model

Change

Delivery

Governance

a) What opportunities are there to strengthen governance arrangements for the NMP? What would these be and why?

Stakeholder engagement

The NMP must include all stakeholders and an open, transparent process encouraging vigorous debate within the realistic context of global and local advances in health. This must be dynamic and include experts in emerging technologies and developing treatments and therapies.

Consumers as part of the governance model

Governance of the NMP can be enhanced by systematic and regular review of the policy through a living guidelines model. As noted, it has been 20 years since implementation, with significant changes in society, investigative technologies and surgical techniques. The Australian society is a health informed community with different and changing expectations from health service delivery. Moving forward with rapid change in health service delivery expected and altered social expectations, regular review of this lynchpin policy is essential.

Change

With expected changes, the move to a fluid policy or living guidelines will ensure relevance to the contemporary environment. This was successfully done during the pandemic, as information became available and necessitated a rapid response to provide measures for the safety of the community. The NMP should be viewed in this way to deliver on stated principles and objectives.

Delivery

Missing from the policy is recognition of and reference to the delivery mechanisms of medicine, treatments, prosthetics, devices and surgical procedures. This needs to be considered for evaluation and identification of gaps in the policy and health service delivery.

Missing from the document is reference to optimum care pathways and clinical guidelines. These play into the implementation and enablers for the quality use of medicines. For genetic, undiagnosed and rare conditions, there may be few documented care pathways or clinical guidelines or evidence that can be referred to for guidance for treatment.

Mechanisms for accountability and transparency need to be considered, developed and evaluated. A policy that has greater relevance to consumers, and that is truly consumer-centric, could improve public conversations.

Governance

Governance infrastructure must include consumers as an equal partner in aspects of policy implementation, monitoring, evaluation and continual improvement. This policy exists to serve the Australian health consumer community and must enable all stakeholders to deliver the best outcomes possible for this community. The governance structure must provide for this and develop as stakeholders develop, knowledge grows, technologies advance, possibilities increase, opportunities arise and the health consumer demands access to this changing health environment.

b) How can communication about the NMP be enhanced or improved?

Improved communication

A diagram of the interconnectedness of the NMP and related bodies and other policies needs to be included in the policy. This will clearly communicate clearly the links and governance arrangements that need to be considered. The proximity of the HTA is critical in the NMP, through to agencies indirectly affected such as the NDIA. Annual reporting on the impacts and influence of the NMP, positives and negatives will inform into currency of the policy.

c) What would be effective mechanisms to support communication about policy?

Timely and accurate communication

Health concerns come to the notice of the public when there is direct and personal experience, sometimes facilitated through the media. A foundation of transparency and communication provides for confidence and trust in health policy and delivery of services when need for treatment arises. For those affected by rare, , undiagnosed and genetic conditions, many have explored options and conducted research enhancing their health literacy, becoming experts in their condition.

Trustworthy

The most trusted source of communication around the NMP for health consumers is going to be informed and knowledgeable other health consumers, particularly when supported by health professionals. This is going to be even more important in coming years as engagement with patients and health consumers builds an ever-increasingly knowledgeable consumer community. The NMP must invest in quality relationships with consumers.

Transparency

Communication strategies for multiple users and stakeholders need to be devised and transparently implemented. It is of concern that that mainstream media and social media can be a significant conveyor of misinformation with little course for redress. Media must be understood as a partner stakeholder, as a responsible stakeholder, who can report accurately on advances made for the community's health.

6. Review the NMP partners and provide options for building greater accountability including addressing conflicts of interest.

Partnerships, partnership values and accountability

The primary stakeholder in an effective NMP is the Australian community, this policy is about them, for them. The means for informing and involvement of health consumers is non-discretionary. Recommendations for engagement are included in this submission.

A documented partnership model with expectations, roles and responsibilities would be an invaluable resource for all stakeholders. This would provide clarity, transparency and engender trust. This partnership model using a living guidelines framework would be most effective with leadership, oversight and evaluation responsibilities clearly articulated in the policy. This would include evaluation and documentation of the implementation of the policy and the gaps and identify new strategies and new partners to address those gaps.

Potential for conflict of interest would be identified, creating expectation and process for declarations and the application of transparent process to manage them ethically, legally and with the health consumers understanding.

Partnership and collaboration that includes the Australian health consumer is the only pathway to more accountable stakeholders (including consumers) and a more effective implementation of the NMP.

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