Identifying priority medicines policy issues for New Zealand; a general inductive study.

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Identifying priority medicines policy issues for New Zealand; a general inductive study

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ABSTRACT

Objectives: To identify priority medicines policy issues for New Zealand.

Setting: Stakeholders from a broad range of healthcare and policy institutions including primary and secondary care

Participants: Exploratory, Semi-structured interviews were conducted with 20 Stakeholders, throughout New Zealand.

Primary and secondary outcome measures: The interviews were digitally recorded, transcribed, coded into INVIVO 10, then compared and grouped for similarity of theme. Perceptions, experiences and opinions regarding New Zealand medicines policy issues were recorded.

Results: A large proportion of Stakeholders appeared unaware of New Zealand's medicines policy. In general, the Policy was considered to offer consistency to guide decision making. Concerns raised were; by whom and how decisions are made and whether desired health outcomes are being measured. Other concerns included; inconsistencies in evidence and across health technologies. Despite attempts to enable equitable access to medicines; lower socioeconomic (including rural residents) Māori and Pacific ethnicities and, rare disorders have continued inequitable access based upon need. Māori had the added issue of higher disease burden and the resultant need for an "inequity lens". Other issues related to physical access, convenience to and affordability of prescribers and, the increase of prescription fees from $3 to $5. Concerns related to PHARMAC included; a constraining budget, non-transparency of in-house analysis, lack of consistency in recommendations between the Pharmacology and Therapeutics Advisory Committee (PTAC) and its subcommittees, its future ability to make autonomous decisions and affordability - with respect to both the Trans-Pacific Partnership Agreement (TPPA) and increases in demand and cost of new medicines. Constraints and inefficiencies in the submission process to access High Cost Medicines also exist.

Conclusion: The results suggest equitable ability for the general population to have funded medicines prescribed. However, vulnerable groups and some procedures still continue to have issues, not necessarily as a direct result of Medicines Policy or PHARMAC.

Strength and limitations of the study

Strengths

- This study is the first independent objective study to identify priority medicines policy issues, from a broad range of Stakeholders.
- There was reasonable satisfaction with the New Zealand Medicines Policy and its principles. In particular that provision of medicines is evidence based, cost effective and there is equitable ability to have prescribed medicines listed as funded, on PHARMAC's schedule.
- Some patient groups still experiencing difficulties in access, particularly groups with rare disorder and the low socio economically oriented; including rural, Māori and Pacifica populations.
- Other medicines policy issues include pharmaceutical industry's pricing of new medicines; medicines registration requirements, submission for funding process, , budgetary constraints for medicines, cultural and health literacy, patient affordability, access to prescribers and the measurement of health outcomes.

Limitations

- The views expressed are from 20 Stakeholders. Issues raised in this research project are therefore indicative. Further research is required to explore the indicative issues.

Introduction

New Zealand has a population of approximately 4.5 million, with a nominal Gross Domestic Product (GDP) of approximately $211 billion(1). Just under 83 percent (82.7%) of health expenditure is publicly funded(2); for those eligible. New Zealand's health and disability budget is $13.983 billion(3). In comparison to other Organisation for Economic Co-operation and Development (OECD) countries, as a percentage of total expenditure on health; New Zealand spends less on pharmaceuticals (2) New Zealanders have an average life expectancy of 81.2 years(2); which is above the OECD average of 80.1(2).

Pharmaceutical Management Agency (PHARMAC) is a separate government agency whose role, is to determine and procure, community and oncology medicines on behalf of the District Health Boards (DHBs) Their scope is now expanding to include hospital medicines and some medical devices. Approximately $795 million and $280 million are available, for procuring community/cancer and hospital pharmaceuticals respectively(4). This compares with a reported estimated spend of $880 million on medical devices(5). Approximately 1848 medicines are subsidised by PHARMAC, for use in the community, mostly accessible via prescription from a medical doctor(6).

For the majority of patients prescribed a medicine listed on PHARMAC's schedule, a $5 District Health Board charge is incurred. For High User or Low socioeconomic patients, access enablers, such as the Community Services and High User cards and now: the Services to Improve Access (SIA) exist to help ease financial burden(6). Additional sources of government funding include; other government agencies such as; Accident Compensation Corporation (ACC) local government, private medical cover and patient "out-of-pocket" co-payments (6).

Medicines and New Zealand

Medicines make a significant contribution to health outcomes(7). In 2007, "Medicines New Zealand" , New Zealand's medicines policy, was launched in response to access concerns from the public(7). The aim of the policy is to promote quality, effective and optimally used medicines. To guide decisions; principles of affordability, equity and need are stated(7).

Literature exist indicating medicines issues for New Zealand related to; inequities in access, affordability, processes used and their funding (8-31). However, no systematic work has been conducted to identify priority medicines policy issues with regards to access and funding of medicines. Within this context, it was considered timely and appropriate to conduct research that could identify priority medicines policy issues for New Zealand.

The dataset obtained from this project was expected to be substantial and provide a solid platform contributing toward informing; medicines policy, expenditure and provision, including the development of optimal medicines management strategies.

Aim

The aim of this project was to identify priority medicines policy issues for New Zealand.

Methods

Study Design and Participant Selection

We conducted a General Inductive study, using semi-structured exploratory interviews during Dec 2012-March 2013. Selection was purposeful; to ensure a broad representation of Stakeholders and their opinions, who had one or more of the following traits in relation to Medicines Policy: involvement in its formation or implementation, had researched and/or commented on Medicines Policy (including having made submissions during its development, n = 10); medically qualified doctor (n = 7; 4 of whom were active prescribers); medicines regulation (n = 1); representation of or, past or current involvement in medicines supply, procurement, funding or provision (excluding dispensing, n = 6); belonging to one of the ethnicities in question (n = 4); involved in medicines management (n = 9); medical information or health technology assessment interest (n = 2); medical interest group representative (past or present, n=4); private health provision and subsidy (n = 1) The participants characteristics are shown in table 1.

A total of 26 Stakeholders were contacted and explained the research involvement. Twenty Stakeholders consented and interviewed. All 20 received a "Participants Information" letter, detailing the involvement, aim and general methods. All signed a confidentiality and anonymity agreement. Fifteen interviews, were conducted face-to-face and five via telephone; due to geographical or time constraints. The median length of interview ranged from 53 – 56 minutes.

Instrument development

The main aim of this research was to identify priority medicines policy areas. An in-depth literature review was conducted to ascertain existing information on pharmaceutical policy. A total of 105 references were identified as useful. The following broad themes were discovered and accordingly sets of questions developed: (1) Medicines Policy; including participant's awareness, description and opinions, (2) Ethnicity inequities in accessing medicines, (3) PHARMAC; its pricing policy, impact upon access, economic modelling, performance, future and any improvements (4)The TPPA; impact upon access and resultant considerations (5) High Cost Medicines access (6) Medicines Policy issues not covered considered important (see appendix one for question details)

The questions were piloted on one Doctor of Māori ethnicity and one Pharmacist; with an interest in Medicines Policy, Medicines Management and Academia, who has previous experience in the Pharmaceutical Industry. Their responses were not included for analysis.

Data Collection

Participants were encouraged to give comprehensive answers. Clarifying and confirming questions were asked where more information was considered necessary, or to avoid interviewer assumption. All participants were thanked for their participation. No gratuity was offered.

All interviews were recorded on a voice recorder, transcribed intelligently; space fillers were omitted to enable ease of reading. Participants received their own transcript to proof, edit and approve. Only the approved editions were entered into INVIVO 10 (QSR International Pty Ltd) for coding.

Coding was conducted two ways. Firstly; categorically according to answers and secondly; highlighted, grouped and compared – according to similarity of theme. Transcripts were checked for any missed issues.

A check for Stakeholder bias was conducted using the coding summaries; no apparent bias was detected. Any variations appeared attributable to Stakeholder knowledge; so were to be expected.

Results

Issues revealed specific to Ethnicity, PHARMAC, the TPPA or High Cost Medicines are reported in those sections. A summary of issues is available in table two.

General Medicines Policy Issues

Nine participants stated they were unfamiliar with the policy. However, four demonstrated a tacit understanding. It was questioned how policy intentions and decisions are made, in the context of being achievable;

> *"How do you attain that?...what is the right way to make those overall policy decisions…" (PI)*

All Participants believed medicines make a positive contribution to health. Differing levels of impact upon health were noted. There was uncertainty as to how the impact is, or could be quantified. The lost opportunity from not capturing and accessing data efficiently, was voiced by 2 Academics for both treatment and outcomes monitoring.

> *"…we are not asking questions about patient health status before and after… so you can really see what is going on, at the GP level. Because that's at least as important as hospitalisation data". (Ac)*

Conversely, one participant said he would prefer to see more investment into epidemiology, as opposed to increasing the medicines budget; in a desire to preserve health.

Low Socioeconomic patients were considered to have a higher burden of disease. Affordability to prescribers was described as the major issue, which may be compounded by the 2013 raise in prescription co-payment from $3 to $5 Australia was contrasted; where there are comparatively low prescriber and higher prescription co-payments.

Despite access enablers, such as the High User Cards and Community Services Cards, it was questioned whether those in need are utilising them. One GP said cost-sensitive patients could be managed with prudent prescribing and education on priorities;

> *"You could get all your medicines for less than a pack of cigarettes. It's educational priorities and various other things, where the effort needs to go rather than reducing the cost much further." (GP)*

The opposite situation of the misuse of access enablers was described;

> *"So it's that whole inverse law ." (GP)*

> *"I initially struggled to understand how somebody could pull up outside a pharmacy in a Mercedes Benz and… present their scripts for their family and handover their Community Services Card… As soon as they get in the country; they put the money into a family trust...So the wealth of the individual gets assessed, which qualifies them for a Community Services Card and then they wave that around." (Ph)*

Sole supply[1] provision raised issues in terms of; supply outages when switching supplier (and having to pay for the alternative option) options for patient intolerance and vulnerability if a significant disruption in supply occurs (eg, a disaster destroying a suppliers warehouse)

There was additional reference to policies and funding needing to be consistent and interlink, especially for priority areas. "Quit-Line" was given as an example; a $40 million funded smoking cessation programme; described as having markedly less evidence than the appropriate medicine (which was not funded for many years) Budgetary constraints were the reason given for this. Equally, the government funding of "alternative medicines" was described as needing debate.

> "*… government is providing funding for people to obtain alternative medicines … real debate to be had… money better spent some place else in the healthcare system?*" *(PI)*

One Doctor voiced frustration at PHARMAC's Therapeutic Advisory Committee (PTAC) [2]being "generalists" who over-ride the recommendation of their subcommittee. With patient sub-typing and genomic medicine on the horizon, he considered "generalists" may not understand what they are assessing and dismiss research; thereby inhibiting access. Access is then through an ability to pay for litigation and decided by a non-medical expert.

With demographic changes increasing demand for healthcare services and a general movement towards increasing costs of new medicines, there was concern for future affordability. Suggestion was funding may move away from being population based, toward funding health outcomes. The biggest concern being the discovery and affordability of a panacea. Academic suggested changes in co-payments, taxation or medicines classification status may result.

The Oncologist had concerns in the availability of future prescribers; possibly compounded by the lack of research to attract them to New Zealand. Extending prescribing ability to non-doctors was considered to help. However, for Oncology, a medical specialist was considered to still be required to make treatment decisions.

Ethnicity Issues

Most issues presented related to Socio-economic variables; which are presented under General Medicines Policy Issues.

Those with poor English speaking skills were described as having access to an English speaking relative or even interpreters if needed.

> *"I think if they can access General Practice or the Hospital system, their access to the medications is just as good as anybody else's. I'm not aware of any specific ethnic problems in accessing our medicines.." (GP)*

[1] Sole supply arrangements are likely to be used by PHARMAC in markets where generic competition exists, resulting in there being only one brand of a particular chemical listed. It is possible that PHARMAC would agree preferred supplier status for some chemicals in exchange for price concessions, affecting access to related pharmaceuticals within the same therapeutic group.32. Pharmaceutical Management Agency. Proposed pricing strategy initiatives - sole supply arrangements. Pharmaceutical Management Agency; 2002 [cited]. Available from: http://www.pharmac.govt.nz/2002/07/19/nhps.pdf.

[2]PTAC is PHARMAC's primary clinical advisory committee. PTAC's role is to provide clinical advice to the Board of PHARMAC.

One GP felt strongly that Māori and Pacifica access inequities are evidenced by poorer health outcomes. He considers his colleagues are treating everyone the same; but with inequitable risk; earlier intervention is required, including improved communication, education and patient engagement;

> *"I think the key issue is the prescribers have a poor understanding of inequalities. Because, the prescribers generally approach things as; I treat everyone the same… they must have an inequity lens on anyone they see… but if the quality of your discussion and the quality in the way in which you prescribed that was poor i.e., you culturally are incompetent and you have a disconnect with the patient…" (GP)*

Other issues related to; Asian ethnicities wanting treatment (Oncology setting) irrespective of likely outcomes and, the use of alternative treatments e.g., St Johns Wort or Vitamin C injections impacting upon medical treatment. One of the doctors had issue with alternative practitioners recommending such treatments as safe and evidence based; upon requesting information to support these treatments, he found the paper to be an out-dated and flawed case study.

Pharmaceutical Management Agency

There was general appreciation shown towards PHARMAC's strategy of creating competition in order to achieve a lower purchasing price. This was seen as advantageous for the purchasing of a greater range of medicines; in the context of a fixed budget.

The budget was defined as the threshold for provision, which was considered too small by an Academic and PI, causing a focus on cost as the driver of value and provision, contributing toward "static efficiency".

> *"If Pharmac's objective is to stay within budget then it's doing well… improve the health of New Zealanders within a capped pharmaceuticals budget…it's doing moderately well…objective were to improve the health of New Zealanders taking into account the financial constraints of Vote Health…it's doing poorly because it should be fighting for a better share of Vote Health." (Ac)*

A Public Servant made the following comment; *"You can always achieve more with more."* In terms of a bigger budget but there isn't an analytical framework in place which would define whether the medicines budget receives a fair proportion of "Vote Health".

There was concern whilst PHARMAC's budget is determined at regular defined intervals, medicines enter the marketplace sporadically; for which funds may not be available.

The Private Health Care Provider(PHCP) thought PHARMAC's approach of requiring new and more expensive medicines to be better than standard medicines a; *"completely acceptable approach."* It was suggested by the PHCP and an Academic that their approach could be more widely adopted; both overseas and with the expansion of PHARMAC's role to medical devices. There was caution given from one pharmacist that PHARMAC's expansion into hospital medicines (in an acute care setting of moribund disease) may limit choices. Concern was shown for risk; if New Zealand is world-leading in this type of provision.

> *"…the expertise PHARMAC has built up…is something that we could learn from and borrow from, for the wider health sector… I'd like to see them take on medical devices, because that is absolutely scandalous that these products are getting onto the market without being properly evaluated…" (Ac)*

Provision was described as having a utilitarian focus; *"The greatest good for the greatest number" (PS)*, which also means you get what you need: not what you want. One Doctor questioned whether the "lost opportunity" from not treating someone is being measured. Rare Disorder patients were mentioned and are discussed under High Cost Medicines.

The distinction was made that provision of a medicine in a cost effective manner; which PHARMAC achieves, is not the same as delivering healthcare.

> *"I find some of their PR a little bit irritating…bray on about the marvellous healthcare they're delivering… delivering medicines in a cost effective manner but that's not saying it's delivering healthcare…" (PHCP)*

The question of the economic modelling Pharmac undertakes received very favourable comments from 15 of the participants. Three participants were not familiar with economic modelling;

> *"I think it's world leading actually. No one else dares do it. That's the crazy thing. Here we are little old New Zealand and we dare do it." (Ac)*

> *"Well I mean, as a tax payer you could argue that for the majority of the products they get in, they've done a really good job of driving cost out of the system." (PI)*

> *"Technically it's very good. PHARMAC considers clinical effectiveness and cost effectiveness.. they make trade-offs… they look at the QALYs and the number of people affected and how their quality of life will be improved and so on, I think is a very good model." (PS)*

It was suggested that cost-benefit should be a consideration; because of valuing the return of an individual back to their normal daily activities, such as what Accident Compensation Corporation does in assessing intervention options.

A Pharmaceutical Industry Representative and Academic were concerned the required economic modelling submitted by suppliers is adjusted with unknown "in-house" variables; making it hard for suppliers to understand decisions. This was contrasted against Medsafe's practice; where decision modelling is transparent;

> *"Pharmac receives a dossier from the company…Assumptions of statistical models get changed…QALYs get changed…population who will use the product get changed…that should be part of a scientific debate…companies don't know what information is being used to make the decisions on their products…we would like a right of reply to those… It happens with MedSafe…Not as though it could potentially negatively affect evidence based decisions."(PI)*

Delays in the submission process of up to 8 years and described as a *"medicines waiting list*" , were of concern for an Academic, Pharmaceutical Industry Representative and Patient Group Representative, who all thought access was related to cost. There was suggestion from one Academic to follow Australia's submission process and out-source assessments from independent bodies.

Trans Pacific Partnership Agreement (TPPA)

Very few participants were familiar with the TPPA, some referred to speculation and no facts. One participant refused to give any comments related to the TPPA.

There was acknowledgement that trade deals are complex and often require compromises and trade-offs. New Zealand was referred to as a *"small country"* and *" we need our trade partners"*. There was concern that already "*big amounts"* are being spent on healthcare and the *"benefits are low"* and if there is a resultant increase in the cost of medicines; where would resources come from; to off-set any cost increases.

The main issues were; (1) Patent extension; delaying generic entry to market, thereby prolonging a higher cost of provision, (2) Industry influencing supply (described as an issue of Sovereignty) may result in quicker access to new medicines but also an increase in public campaigns and appeals processes if PHARMAC's decision are unpopular with the industry or patient groups. (3) The call for transparency in PHARMAC's assessment process caused the most concern and confusion. One Academic said he didn't think PHARMAC could be more transparent and that transparency might mean the industry discloses its pricing processes and the results of all clinical trials.

In general, scepticism was voiced as to what the driving force is behind the agreement and what the benefits would be for New Zealand; with the USA being a protected market (heavily subsidised) Australia was described by a Pharmaceutical Industry Representative, as getting "trounced" over their agreement with the USA; losing a lot of their pharmaceutical production and jobs as a result;

> *"Forget it…wouldn't even bother going along to the negotiations"(PHCP)* or; *"tell the US to bugger off quite frankly. You either put everything on the table and we talk about it or no, you don't…We should learn from what happened in Australia…" (PI)*

Conversely, another participant suggested whilst "America" has influence, it may become limited as a result of the influence of China's developing economy and differing ideas around protection and, new opportunities may develop:

> *"…a hugely developing economy in the form of China that basically has total disregard for such things… so the ability for America…is probably going to be limited in the world of the future, and maybe different forms of protection of ideas will kind of evolve… it's very hard to predict how the market might respond or what kind of new opportunities develop." (PS)*

One Academic suggested that PHARMAC's monopsony is an anathema to the USA. A Pharmaceutical Industry Representative said Medicines New Zealand (New Zealand's prescription medicines representative association; same title as the policy) is attempting to ensure its USA equivalent understands New Zealand's medicines system;

> "*…working quite hard to ensure…our sister organisation in the US is effectively asking the US government to achieve out of the process, is well enough informed to understand actually what the New Zealand model does achieve, what it doesn't achieve and how that can be improved…So we're working hard to make sure it's a process that actually benefits New Zealanders as well, and all of the transparency, timeliness, appeals – those aspects that we've discussed, are exactly I think what the US is likely to be asking for.*

Most considered that New Zealand's current ability to; access generic medicines or, independence in procuring medicines should be upheld. If not, funds may need to be redirected from other services or, patient co-payments would need to rise, in order to compensate a likely increase in the cost of medicines.

High Cost Medicines

A GP questioned the necessity of continuing the Special Authority (SA)[3] status for a medicine, once the appropriate use of a medicine has been established. Not all participants were familiar with the Named Patient Pharmaceutical Assessment (NPPA)[4] access scheme. Most High Cost Medicines were described as being *"breakthrough"* or *"expensive"; which* are restricted, to control spending. One participant said, if it was "dirt cheap", there would be "no argument" indicating the case, even if the medicine didn't have clear health benefits*;*

> *"My bet, is that PHARMAC would listen to anyone that agrees with them saying no. Because it's expensive ....They are diametrically opposed for a reason and the reason is cost." (Ph)*
>
> *"The Rabbits in charge of the lettuce patch" (M)*

One Public Servant thought there to be no inappropriate blocking of access to medicines, as no complaints about access have been received at their level. Equally another Public Servant commented that there are patients accessing medication costing up to $500, 000 dollars per year;

> *"…So it's not that the system can't cope with treatments that are high cost, it's just that we would expect a return for that cost and for it to be justifiable in terms of what we value." (PS)*

A small group of patients were described as not having access to high cost medicines. Access was described as; "the collective good". Conversely; *"people dying from a lack of access to very cheap and simple therapies"* were described. It was suggested that it is the DHBs remit to look after its population, highlighting the issue of population versus individual access. A statement was made; are we advocating treatment at any cost and if so, who pays?

> *"It's a question of who pays for all these things. I think if you have pretence; like there is in the USA, that cost isn't of any relevance… then you're going down the wrong path." (PHCP)*

The Oncologist described the NPPA process as inefficient; a comprehensive and referenced application, takes him up to 6 hours; potentially impacting on his clinic time and perversely hindering patient access. He suggested PHARMAC at a nominal cost could employ someone to aid in information gathering and in the process develop expertise.

Equally the Oncologist believes oncology has the stigma that everyone dies; but individual survival may be greater than the median survival assessment. This issue was presented in comparing the availability of 2-3 drugs in Australia; unavailable in New Zealand.

Questions were posed; (1) Is it fair to give 4th or 5th line chemotherapy and not give a first line treatment e.g. for Rare Disorders? and, (2) When do you stop treatment; a patient was described as gaining access to expensive medication, their condition was fragile and they died a few weeks later.

[3] Special Authority criteria define the clinical circumstances of patients who can receive funding for the medicine. People may first be required to try a less expensive medicine or the medicine may need to be prescribed by a particular type of health practitioner.

[4] NPPA is a mechanism to give individual named patients access to medicines they need, but which aren't funded on the Pharmaceutical Schedule. NPPA replaces the three Exceptional Circumstances (EC) schemes that PHARMAC previously managed.

> *"I think it's important if Pharmac has a few loose strings in terms of hospital and severe rare conditions. They are perhaps because of how they are funded, they want a very narrow perspective on those, to try and avoid blow out. They are very emotive issues we don't always know how to best manage people's care. "* (Ph)

High need patients, such as Rare Disorder Patients received the most sympathy for difficulty in access because of the exclusion criteria. Evidence requirements were described as difficult to attain due to low patient numbers. Conversely, the PHCP suggested the supplier needs to produce quality evidence;

> *"…where there's some evidence, but not solid or quality evidence: … the company doing the – providing the medication, it behoves on them to do some research in those areas and produce quality data."(PHCP)*

Discussion

We purposefully attempted to be open to issues and their capture, despite some issues already being identified. Our focus was on access to medicines. It is possible there are other issues in existence, we neither recognised nor captured. We did not seek to determine issues specifically related to generic medicines; considered a "vital component of New Zealand's medicine cost management policies", by Babar et al(12); apart from the sole supply issues (which encompasses generic medicines) a lack of palatability was additionally reported, from a brand of paracetamol not being coated.

Medicines Policy

The significance of the lack of familiarity shown with "Medicines New Zealand" is uncertain but better familiarity with policy and processes of evaluation may be required if the goals are to be fulfilled.

Medicines are clearly valued health interventions; evidenced from the budget, literature and responses from Stakeholders. The smaller percentage spend on pharmaceuticals in New Zealand (described as a constrained budget) compared with similar countries such as Australia, UK and USA(2) , may in fact reflect the price reduction strategies that are implemented by Pharmac; as opposed to less opportunity to improve health outcomes. However, this needs to be tested through robust research on health outcomes and their relationship to pharmaceutical spending.

Delayed access and the resultant impact discussed by some of the participants was also described by Eliis and Hamer(33) in relation to statin availability for atherosclerotic patients as probably negatively impacting health outcome and considered to be due to the capped budget. They considered this "anomalous", as other types of health care are not capped. This anomaly was also described by a number of participants but may change with PHARMAC's expanding role.

New medicines are increasing in costs along with demand, causing tension in affordability. Price efficiency initiatives, such as what PHARMAC encourages, help ease the tension in affordability of provision. Another option is to reduce demand; either through gate keeping (not usually a popular choice) or genuine effects, such as initiatives to maintain health and ameliorate or prevent disease. We assume a reduction in demand and therefore burden of provision, will result in healthcare becoming more affordable, for providers and helping those remaining in need.

Resolving disputes between the experts and provision were described as being dependent upon an individual's ability to pay for litigation; not an equitable process and one where a non-medical expert makes

the decision. Manning(34) discussed resolving issues may benefit from a disputes panel (funded by the Ministry of Health) comprising a broad range of experts in scientific, economic, policy and ethical evaluation, to provide an objective decision. Manning additionally reported; approximately 1/6 of the United Kingdom's National Institute for Healthcare and Clinical Excellence (NICE)(35) recommendations are appealed and upheld. There shouldn't be great demand, if evaluation processes are robust.

Ethnicity

Our participants revealed that low socioeconomically related populations (encompassing Māori and Pacifica people) are continuing to have access issues related to financial, structural, educational and cultural barriers. These findings were consistent with that of Jatrana et al(36); who found Māori and Pacific people were more likely to defer purchasing a prescription due to cost, which at that time was $15. Māori represent approximately 15% of New Zealand's population(37) and on average have the poorest health status of any ethnic group in New Zealand(38, 39).

He Korowai Oranga: the Māori Health Strategy (2002)(38), recognises the Treaty of Waitangi principles of; partnership, participation and protection, through which, the aim is to reduce existing health inequalities. This aim is extended to include the Pacific people who represent 6.5% of the population and also experience health inequalities. Like Māori, they are over represented by a low socioeconomic situation, reflecting low affordability and health literacy, which in turn affects access. Ministry of Health initiatives; Whanau Ora: to build the health, participation and capability of families and, One Heart Many Lives: to improve the cardiac health of Māori and Pacific men, along with recent changes in health practitioner training, appear good initiatives for engaging Māori and Pacifica in a culturally appropriate way. It would be prudent to evaluate their impact.

Jatrana et al's(40); 2009 analysis, found overall 15.5% of the 18320 respondents analysed had deferred seeing their doctor and 6.4% buying a prescription, within the preceding 12 months because of cost; prescription fees were still $15. Jatrana et al(40) (possibly due to differences in methodology from our study) additionally found; younger populations, smokers, those unmarried or experiencing high levels of psychological distress, or with more than 2 co-morbidities also deferred doctor visits and buying a prescription. The Ministry of Health recently launched; Services to Improve Access (SIA)(41) – an additional targeted capitation payment, available to Primary Healthcare Organisations to reduce health inequalities. It is designed for new services (eg, outreach programmes) or improving access (eg funding transport) for Māori, Pacific people and those of low socioeconomic status. Once SIA is embedded, it would be prudent to evaluate its impact.

We did not have any issues specifically described for new immigrants. It was described to us that that patients with poor English speaking capability, present to practitioners with an English speaking person, or frequent a surgery of their ethnicity. This is at odds with Babar et al(9); who found for 11 Chinese and Indian migrants, residing in New Zealand for less than 5 years; financial barriers existed in affording doctors, pharmacists and medicines; their preferred traditional medicines were also difficult to obtain in New Zealand. Babar additionally found there is a lack of information on New Zealand's medicines system, provision, classifications and language barriers. This anomaly may highlight the differences in perspective and experience of the Stakeholders we interviewed.

Backman et al(42), propose ethnicity as one of five priority indicators for vulnerability and discrimination, which may affect the accessing of health-related services and therefore pose a risk of reduced health. The

United Nations and World Health Organisations, when discussing the Right to Health(43); refer to migrants as being vulnerable to reduced access to health services for reasons that include; language or cultural barriers. New Zealand has a significant migrant population, reported as 927 000 in 2006(44). The current main countries for immigration are: China (15%), United Kingdom (unspecified), India (13%) and the Philippines (8%)(44) Asia and India have different medicines access systems to New Zealand. As a result of these immigration statistics and Babar et als work, there may be significant issues for people from such countries not being familiar with New Zealand's health system, resulting in difficulties in accessing medicine(8-10). In light of this, it may be worth investigating new immigrant issues further.

PHARMAC

The general appreciation for the need to be efficient to provide more medicines expressed by our Stakeholders, was also shown by Ragupathy et al(26). Included, was the need to apply consistent economic evaluations to other health technologies; to support congruous decisions for resource allocation. PHARMAC's expansion into procuring hospital medicines and medical devices may enable greater consistency of evaluation across technologies.

The significance of PHARMAC's role expansion should not be underestimated. PHARMAC will need to practice caution in expanding their role into hospitals, which are generally settings of acute and moribund disease. We are unaware whether a closed formulary has occurred elsewhere in the world. PHARMAC have been noted to have consulted directly with Medical Specialist groups to discuss their role expansion, including consultation on hospital medical devices(45), so would appear to be fully cognisant and appreciative of this issue.

We found delays of up to 8 years, in PHARMAC's process for funding medicines onto the Pharmaceutical Schedule, which our Stakeholders purported to be due to the medicine's pricing and/or PHARMAC's budget not being able to expand. Other reasons may be the medicine's priority status, insufficient information or, not meeting PHARMAC's nine decision criteria(46). The question is whether this means delays in therapeutic advancement.

The measurement of opportunity foregone was of clear concern to the Oncologist we interviewed. New Zealand has a capped medicines budget; it cannot expand and therefore drives the need for efficient spending (determined using cost utility analysis; where medicines are assessed against QALY gains per $1 million) Using this process for provision means there is opportunity foregone, as described by Milne and Wonder(14). We are not aware of any research assessing opportunity foregone or, other Specialist viewpoints on access, except Ellis and Hamer(33); in 2008 discussing the delayed availability of Cardiac medicines, MacCormack et al(31); in 2009, assessing Stakeholders views on needed access to High Cost Medicines and The Sage report for the Ministry of Health(22); in 2010 reporting the consultation of Stakeholders on the proposal to expand PHARMAC's role.

Sole supply issues (supply outages, lack of palatable formulations, resultant out of pocket payments for alternatives and vulnerability as a result of a disaster) were reported as still continuing, despite there being penalties for suppliers. This was also reported by Babar et al(47), who reported additional concerns with poor quality products in the past from previous studies.

Trans-Pacific Partnership Agreement (TPPA)

There is very little information available on the TPPA, the reason given; particulars of the negotiation are changing. What does exist; concurs with our findings; it questions the motivation and self-interest of parties involved and warns of possibly binding impacts that may affect health services budgets and, PHARMAC's autonomy including method of procurement and provision.(25-29, 48) Such impacts stemming from the USA's desire for stricter protection of intellectual property rights, transparency of in-house evaluation, regulatory coherence, dispute settlement, government procurement and evidence based decisions being contestable in court. Unless budgets are expanded to cope with likely increases in costs, there may need to be a re-evaluation of provision, subsidies and co-payments. In contrast to existing publications, our research additionally suggested a TPPA may enable earlier access to newer medicines. It may be of use to quantify what the effect of a TPP would have upon medicines and how it could be dealt with.

New Zealand has an open economy and has a number of existing trade agreements but often encounters trade barriers overseas. There is an implied concern that trade agreements have proliferated worldwide and there is a competitive need to join in, or risk being disadvantaged(29).

The WHO(43) references campaigns by various human rights Non-Government Organisations (NGOs) which have raised the awareness of possible negative implications of international trade agreements on the price of new essential medicines. Access to essential medicines has become an indicator for governmental commitment to the right to health.

High Cost Medicines

A significant issue discussed in our study; was the need to differentiate between high cost medicines and highly specialised needs or medicines in relation to the NPPA access scheme. McCormack et al (31), suggest a medicine that costs $20 000 per patient per year may be considered high cost. It is important to be cognisant of the total cost to the health system of any medicine; which is dependent upon the number of patients treated (volume used) and the acquisition price. Some high cost medicines may not result in a high total cost to PHARMAC, for some patient groups. Gallego et al(49), question how treating large populations at high total cost for small population gains, compares with treating smaller numbers of patients for possibly significant benefit.

The issue of treating large versus small populations, may intensify with patient subtyping and genomic medicines development, as described by the Oncologist; where greater expectation to fund (ie, demand) may occur. With the NPPA process now reported in our findings, to enable cancer patient subtyping information; cancer medicines outcomes may become easier to measure and if positive, make it harder to decline funding treatments. It may also mean the table is turned and large populations end up having limited treatment options, if outcomes cannot be measured in the same way. Equally, funding outcomes will give a clear indication for innovation and direction to both suppliers and funders of medicines.

Our findings describe both the SA and the NPPA access schemes as being inefficient. The SA inefficiency finding is also supported by Babar et al(47). Once correct prescribing of a medicine has been established it may not be necessary to continue a medicine's SA status. The NPPA process appears to impact significantly on consultant clinic time; which may perversely hinder patient access. With demographic trends indicating greater demand for such medicines, the impact of the inefficiency may intensify. PHARMAC's website lists 555 approvals and 15 declines for NPPA access(50). It may be more efficient for PHARMAC, at a nominal cost, to contract an evidence based facilitator, to ease the burden of application for clinicians.

Difficulty in access for high cost medicines, including Rare disorder patients, as described by our participants, has been widely documented(31, 49, 51-60) With delays in the Scheduling process (described in years by our Stakeholders) Rare diseases access using PHARMAC's criteria may be significantly hindered. The nature of Rare diseases, makes it hard to gain the necessary evidence PHARMAC requires for evaluation. This issue is compounded for suppliers because the need to satisfy both manufacturer ordering and regulatory requirements, adds to the unit cost of supply for low volume demand medicines. It may be worth investigating options to reduce cost of supply and provision in the context of constrained evidence. PHARMAC have recently sought public and professional input into its decision criteria. The results have yet to be published but may reveal new options or initiatives.

Our research highlighted the issue of access to medicines of therapeutic value in the context of a fixed predetermined budget and the difficulties in how priorities for funding are determined. Yu et al(61), discussed the issue of having equal need requires equal opportunity to access care and suggest where evidence requirements are not achieved; treatment commence on a trial and outcome basis. This does come with ethical concerns but may enable both access and capturing evidence. MacCormack et al(31), suggest "risk sharing" supply to ensure some form of access; defining a threshold for maximum numbers to treat for a high cost medicine, above which, the supplier funds.

Conversely, Simoens et al(54) caution providing access to medicines with limited effectiveness; implies rare disorders health improvement is more valuable than a common disease, which challenges the utilitarian view of; the health gain of each patient is valued equally. With increasing effort in the development and resultant increase in the availability of orphan drugs; this issue may only worsen. Equally other questions arise; because we see the ill health can't mean preferential treatment over someone who has a "silent" state of declining health. There are people not getting access to inexpensive medicines; who are at risk; as stated by an Academic. Perhaps remedying issues of access based on need, could start with prioritising based on the impact of an unmet need?

A new medicine does not necessarily mean improved therapeutic value. Australia is our natural comparator country. Vitry et al(62), recently evaluated the therapeutic value of 217 recommendations by the Australian Drug Evaluation Committee, publicly available between 2005 and 2007, using the Motola and Ahlqvist-Rastad rating scores. Sixty nine were for new medicines, 55 were evaluated, four of which had 2 indications; making a combined total of 59 new drugs/indications. Most indications were for serious diseases (91.5%), 5.1% for risk factor management and 3.4% for non-serious diseases, for which treatments already existed. Between 32.2% and 47.5% were rated as therapeutic innovations. Vitry et al(62) highlighted that a new medicine may not equate to an improvement in therapeutic value, which is ultimately what PHARMAC seeks and may help explain differences in numbers of medicines funded between the two countries. It was also noted that, there is no standard methodology for evaluating the therapeutic value of new medicines.

Conclusion

Overall, despite issues being identified, there was reasonable satisfaction with the New Zealand Medicines Policy and its principles. In particular that provision is evidence based, cost effective and there is equitable ability to have prescribed medicines listed as funded, on PHARMAC's schedule.

However, despite this, there appears to be some patient groups still experiencing difficulties in access, not necessarily appearing as a result of Medicines Policy or PHARMAC. Such groups being; Rare disorder and the Low socio economically oriented; including rural, Māori and Pacifica populations. Other issues ranged from the pharmaceutical industry's pricing of new medicines; as well as manufacturer and registration requirements, the submission for funding process, increasing demand and costs, budgetary constraints, cultural and health literacy, patient affordability and access to prescribers, through to knowledge development for clinical expertise and the measurement of health outcomes.

Our study has highlighted issues in access based upon need and the consequences of unmet need. The context of provision being based upon a fixed predetermined budget and increasing demand causing constraints in affordability. We suggest these issues and consequences of unmet needs may worsen and options for demand and provision may need to be explored further.

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Ethics: Ethics approval was obtained from The University of Auckland, Ethics Committee. Approval number; 8367

Authors contribution: ZB conceptualised and designed the study. The data collection, entry and analysis was handled by SF and ZB. SF and ZB wrote the manuscript. The final version is approved by all authors. ZB acts as an overall guarantor to this study.

Transparency: ZB affirms that the manuscript is an honest, accurate, and transparent account of the study being reported; that no important aspects of the study have been omitted.

Provenance and peer review: Not commissioned; externally peer reviewed.

Data sharing statement: The original data are available from the principal author (ZB).

Table one: Stakeholder Characteristics:

Stakeholder Group	Number (n)	Area of Professional Group/Title (n)
Academia (Ac)	3	Sociologist (2) Pharmacoeconomist (1)
Public Service (PS)	5	Politician (1) Medsafe (1) Policy Analyst (1) DHB Planning (1) Pharmac (1)
Medicine (M)	4	Oncologist (1) General Practice (3)
Pharmacist (Ph)	3	DHB (2) Community based(1)
Pharmaceutical Industry (PI)	2	Manufacturing (1) Representative (1)
Patient Group Representative (PGR)	2	Long Term Conditions (2)
Private Health Care Organisation (PHCO)	1	Medical doctor (1)

References

1. Statistics New Zealand. Top Statistics. 2013 [cited]. Available from: http://www.stats.govt.nz/browse_for_stats/snapshots-of-nz/top-statistics.aspx.
2. Organisation for Economic Co-operation and Development countries. OECD Health Data 2013 - frequently requested data: health expenditure. 2013 [cited]. Available from: http://www.oecd.org/els/health-systems/oecdhealthdata2013-frequentlyrequesteddata.htm.
3. Ministry of Health. Funding. Ministry of Health; 2013 [cited 2013]. Available from: http://www.health.govt.nz/new-zealand-health-system/funding.
4. Pharmaceutical Management Agency. Setting and managaing the combined pharmaceutical budget (CPB). New Zealand Government; 2013 [updated 2013; cited 2013]. Available from: http://www.pharmac.health.nz/ckeditor_assets/attachments/381/infosheet-08-community-pharm-budget-2013.pdf.
5. Pharmaceutical Management Agency. PHARMAC and hospital medical devices. 2013 [cited]. Available from: http://www.pharmac.health.nz/medicines/hospital-devices/.
6. Pharmaceutical Management Agency. New Zealand Pharmaceutical Schedule. 2013 [updated April, 2013; cited 2013]. Available from: http://www.pharmac.govt.nz/2013/04/03/Sched.pdf.
7. Associate Minister of Health, Minister of Health. Medicines New Zealand: Contributing to good health outcome for all New Zealanders. In: Health Mo, editor. Wellington, New Zealand: Ministry of Health, Wellington, New Zealand; 2007.
8. DeSouza R, Garret N. Access Issues for Chinese People in New Zealand Final Report. : Auckland University of Technology, Auckland; 2005 2005. Contract.
9. Babar ZUD, Pengelly K, Scahill SL, Garg S, Shaw J. Migrant health in New Zealand: Exploring issues concerning medicines access and use. Journal of Pharmaceutical Health Services Research. 2013;4(1):41-9.
10. Suin T. An holistic approach to Asian health Fourth internaitonal Asian health and well-being conference; 2010; Auckland. 2010.
11. Ragupathy R, Aaltonen K, Tordoff J, Norris P, Reith D. A 3-dimensional view of access to licensed and subsidized medicines under single-payer systems in the US, the UK, Australia and New Zealand. PharmacoEconomics. 2012;30(11):1051-65.
12. Babar ZUD, Grover P, Stewart J, Hogg M, Short L, Seo HG, et al. Evaluating pharmacists' views, knowledge, and perception regarding generic medicines in New Zealand. Research in Social and Administrative Pharmacy. 2011;7(3):294-305.
13. Wonder M, Milne R. Access to new medicines in New Zealand compared to Australia. New Zealand Medical Journal. 2011;124(1346):12-28.
14. Milne R, Wonder M. Response to PHARMAC on access to new medicines in New Zealand compared to Australia. New Zealand Medical Journal. 2011;124(1347):18.
15. Tordoff JM, Norris PT, Reith DM. "Price management" and its impact on hospital pharmaceutical expenditure and the availability of medicines in New Zealand hospitals. Value in Health. 2008;11(7):1214-26.
16. Aaltonen K, Ragupathy R, Tordoff J, Reith D, Norris P. The impact of pharmaceutical cost containment policies on the range of medicines available and subsidized in Finland and New Zealand. Value in Health. 2010;13(1):148-56.
17. Cumming J, Mays N, Daubé J. How New Zealand has contained expenditure on drugs. BMJ (Online). 2010;340(7758):1224-7.
18. Metcalfe S, Grocott R. Comments on "simoens, s. health economic assessment: A methodological primer. int. j. environ. res. public health 2009, 6, 2950-2966"-New Zealand in fact has no cost-effectiveness threshold. International Journal of Environmental Research and Public Health. 2010;7(4):1831-4.
19. Moodie P, Metcalfe S, Poynton M. Do pharmaceutical score cards give us the answers we seek? New Zealand Medical Journal. 2011;124(1346):69-74.

20. Moodie P, Metcalfe S, McNee W. Response from PHARMAC: Difficult choices. New Zealand Medical Journal. 2003;116(1170).
21. Grocott R. Applying Programme Budgeting Marginal Analysis in the health sector: 12 years of experience. Expert Review of Pharmacoeconomics and Outcomes Research. 2009;9(2):181-7.
22. Sage D. Report on the consultation period for the proposal to expand the functions of PHARMAC; 2010. Contract.
23. Morgan S, Boothe K. Prescription drug subsidies in Australia and New Zealand. Australian Prescriber. 2010;33(1):2-4.
24. Carlson JJ, Sullivan SD, Garrison LP, Neumann PJ, Veenstra DL. Linking payment to health outcomes: A taxonomy and examination of performance-based reimbursement schemes between healthcare payers and manufacturers. Health Policy. 2010;96(3):179-90.
25. Gleeson D, Lopert R, Reid P. How the Trans Pacific Partnership Agreement could undermine PHARMAC and threaten access to affordable medicines and health equity in New Zealand. Health Policy. 2013.
26. Ragupathy R, Tordoff J, Norris P, Reith D. Key informants' perceptions of how pharmac operates in New Zealand. International Journal of Technology Assessment in Health Care. 2012;28(4):367-73.
27. Moynihan R. New Zealand agency comes under pressure to pay more for drugs. BMJ (Clinical research ed). 2011;342.
28. Foster RH, Wilson N. Trans-pacific partnership: Will Pharmac become a victim of its own success? BMJ (Online). 2011;343(7818).
29. Faunce TA, Townsend R. The Trans-Pacific Partnership Agreement: challenges for Australian health and medicine policies. Medical Journal of Australia. 2011;194(2):83-6.
30. Raftery JP. Paying for costly pharmaceuticals: regulation of new drugs in Australia, England and New Zealand. Medical Journal of Australia. 2008;188(1):26-8.
31. MacCormack P, Quigley J, Hansen P. Review of access to high-cost, highly specialised medicines in New Zealand. ; 2010. Contract.
32. Pharmaceutical Management Agency. Proposed pricing strategy initiatives - sole supply arrangements. Pharmaceutical Management Agency; 2002 [cited]. Available from: http://www.pharmac.govt.nz/2002/07/19/nhps.pdf.
33. Ellis CJ, Hamer AW. Cardiovascular health in New Zealand: areas of concern and targets for improvement in 2008 and beyond. New Zealand Medical Journal. 2008;121(1269):5-10.
34. Manning J. Priority-setting processes for medicines: the United Kingdom, Australia and New Zealand. Journal of Law & Medicine. 2011;18(3):439-52.
35. National Institute for Health and Care Excellence, . [cited]. Available from: http://www.nice.org.uk.
36. Jatrana S, Crampton P, Norris P. Ethnic differences in access to prescription medication because of cost in New Zealand. Journal of Epidemiology and Community Health. 2011;65(5):454-60.
37. Statistics New Zealand. Māori population estimates. 2012 [cited]. Available from: http://www.stats.govt.nz/browse_for_stats/population/estimates_and_projections/maori-population-estimates.aspx.
38. Ministry of Health NZG. He Korowai Oranga: Māori Health Strategy, . 2002 [cited]. Available from: http://www.health.govt.nz/publication/he-korowai-oranga-maori-health-strategy.
39. Ministry of Health NZG. The Health of Māori Adults and Children. 2013 [cited]. Available from: http://www.health.govt.nz/publication/health-maori-adults-and-children.
40. Jatrana S, Crampton P. Primary health care in New Zealand: Who has access? Health Policy. 2009;93(1):1-10.
41. Ministry of Health NZG. Services to improve access. 2011 [cited]. Available from: http://www.health.govt.nz/our-work/primary-health-care/primary-health-care-services-and-projects/services-improve-access.
42. Backman G, Hunt P, Khosla R, Jaramillo-Strouss C, Fikre BM, Rumble C, et al. Health systems and the right to health: an assessment of 194 countries. The Lancet. 2008;372(9655):2047-85.
43. The United Nations WHO. The right to health. 2008 [cited]. Available from: http://www.who.int/hhr/activities/Right_to_Health_factsheet31.pdf.

44. Ministry of Business Innovation and Employment. New Zealand Government. Migration research. [cited]. Available from: http://dol.govt.nz/research/migration/monthly-migration-trends/13jun/index.asp.
45. Pharmaceutical Management Agency. Adapting the PHARMAC model for medical devices management,. 2013.
46. Pharmaceutical Management Agency. Decision criteria Pharmac; 2013 [cited 2013 11.4.13]. Available from: http://www.pharmac.health.nz/medicines/how-medicines-are-funded/decision-criteria.
47. Babar ZUD, Grover P, Butler R, Bye L, Sheridan J. A qualitative evaluation of general practitioners' perceptions regarding access to medicines in New Zealand. BMJ Open. 2012;2(2).
48. Kelsey J. Hidden agendas what we need to know about the TPPA. BWB texts; 2013 [cited. Available from: www.bwb.co.nz.
49. Gallego G, Taylor SJ, Brien JaE. Funding and access to high cost medicines in public hospitals in Australia: Decision-makers' perspectives. Health Policy. 2009;92(1):27-34.
50. Pharmaceutical Management Agency. NPPA outcome report cumulative for March - December (updated 24/1/13). Pharmaceutical Management Agency,; 2013 [cited]. Available from: http://www.pharmac.health.nz/ckeditor_assets/attachments/249/summary_of_nppa_decisions_dec_12.pdf.
51. Forman J, Taruscio D, Llera VA, Barrera LA, Coté TR, Edfjäll C, et al. The need for worldwide policy and action plans for rare diseases. Acta Paediatrica, International Journal of Paediatrics. 2012;101(8):805-7.
52. Lu CY, Williams K, Day R, March L, Sansom L, Bertouch J. Access to high cost drugs in Australia. British Medical Journal. 2004;329(7463):415-6.
53. Lee TH, Emanuel EJ. Tier 4 drugs and the fraying of the social compact. New England Journal of Medicine. 2008;359(4):333-5.
54. Simoens S, Cassiman D, Dooms M, Picavet E. Orphan drugs for rare diseases: Is it time to revisit their special market access status? Drugs. 2012;72(11):1437-43.
55. Munoz J, Kurzrock R. Targeted therapy in rare cancers-adopting the orphans. Nature Reviews Clinical Oncology. 2012;9(11):631-42.
56. Hudson I, Breckenridge A. The challenges of orphan drugs and orphan diseases: Real and imagined. Clinical Pharmacology and Therapeutics. 2012;92(2):151-3.
57. Drummond MF, Wilson DA, Kanavos P, Ubel P, Rovira J. Assessing the economic challenges posed by orphan drugs. International Journal of Technology Assessment in Health Care. 2007;23(1):36-42.
58. Joppi R, Bertele V, Garattini S. Orphan drugs, orphan diseases. The first decade of orphan drug legislation in the EU. European Journal of Clinical Pharmacology. 2013;69(4):1009-24.
59. Largent EA, Pearson SD. Which orphans will find a home? The rule of rescue in resource allocation for rare diseases. Hastings Center Report. 2012;42(1):27-34.
60. Cheema PK, Gavura S, Migus M, Godman B, Yeung L, Trudeau ME. International variability in the reimbursement of cancer drugs by publically funded drug programs. Current Oncology. 2012;19(3):e165-e76.
61. Lu CY, Macneill P, Williams K, Day R. Access to high cost medicines in Australia: Ethical perspectives. Australia and New Zealand Health Policy. 2008;5.
62. Vitry A, Shin N, Vitre P. Assessment of the therapeutic value of new medicines marketed in Australia. Journal of Pharmaceutical Policy and Practice. 2013;6(1):2.

Appendix One

Questionnaire

1. Medicines Policies
   - What contribution medicines make to the health status of New Zealand(ers) ?
   - Awareness of "Medicines New Zealand"; the New Zealand Medicines Strategy ?
   - The impact of Medicines Policy upon access to medicines ?
   - What if anything, could be done to improve Medicines Policy; Why and How?

2. Medicines Access and Inequalities/Inequities on the basis of Ethnicity
   - What is their view of medicines access and inequalities based on; the identified ethnicities ?
   - What if anything, could be done to improve access and inequalities for these ethnicities ?

Note: the ethnicities were described as: Māori, Pacifica, Indian or Asian, or such people where English may not be a first language

3. Pharmac
   - Awareness of Pharmac's pricing policy ?
   - Description of Pharmac's pricing policy ?
   - Awareness of how Pharmac subsidises and funds medicines ?
   - Pharmac's impact upon access ?
   - Opinion of Pharmac's model of pricing in terms of cost effectiveness, cost utility and reference pricing ?
   - How well Pharmac is performing it's role, what impact has it had ?
   - What is the future for Pharmac, in next 5,10,20 years. What could be the likely issues ?
   - What if anything, could be improved in relation to Pharmac ?
   -

4. Transpacific Partnership Agreement (TPP)
   - With the likely TPP agreement with United States of America; what impact will it have on medicines procurement and availability and, why ?
   - What needs to be considered with the TPP and access to medicines ?

5. Accessing and Funding of High Cost Medicines
   - Awareness of the accessing and funding of High Cost Medicines and opinion of the process ?
   - Impact of Medicines Policy upon access to High Cost Medicines ?
   - What improvements could be made in the accessing and funding of High Cost Medicines?

Note: a description of high cost medicines was given, such as; beyond the average person's ability to afford e.g., some oncology and Rare Diseases medicines

6. Supplementary Questions
   - Have the above questions covered Medicines Policy ?
   - Any other aspects of Medicines Policy affecting access, not covered ?
   - Will the current system of medicines access continue, or not ?
   - What is the future for Medicines Policy ?

Anything else to say in relation to Medicines Policy and the accessing of medicines ?

Consolidated criteria for reporting qualitative studies (COREQ): 32-item checklist

Developed from:
Tong A, Sainsbury P, Craig J. Consolidated criteria for reporting qualitative research (COREQ): a 32-item checklist for interviews and focus groups. *International Journal for Quality in Health Care*. 2007. Volume 19, Number 6: pp. 349 – 357

YOU MUST PROVIDE A RESPONSE FOR ALL ITEMS. ENTER N/A IF NOT APPLICABLE

No. Item	Guide questions/description	Reported on Page #
Domain 1: Research team and reflexivity		
Personal Characteristics		
1. Inter viewer/facilitator	Which author/s conducted the inter view or focus group?	Susan Francis
2. Credentials	What were the researcher's credentials? E.g. PhD, MD	RN, PG Dip
3. Occupation	What was their occupation at the time of the study?	Research Assistant
4. Gender	Was the researcher male or female?	Female
5. Experience and training	What experience or training did the researcher have?	Qualitative, NVivo
Relationship with participants		
6. Relationship established	Was a relationship established prior to study commencement?	An email was sent to introduce the objective and scope of the study
7. Participant knowledge of the interviewer	What did the participants know about the researcher? e.g. personal goals, reasons for doing the research	An interest to conduct research on NZ medicines policy issues
8. Interviewer characteristics	What characteristics were reported about the inter viewer/facilitator? e.g. Bias, assumptions, reasons and interests in the research topic	Stakeholder characteristics are described in table 1.
Domain 2: study design		
Theoretical framework		
9. Methodological orientation and Theory	What methodological orientation was stated to underpin the study? e.g. grounded theory, discourse analysis, ethnography, phenomenology, content analysis	Content analysis General inductive approach
Participant selection		
10. Sampling	How were participants selected? e.g. purposive, convenience, consecutive, snowball	Purposive

11. Method of approach	How were participants approached? e.g. face-to-face, telephone, mail, email	Email, face to face, through telephone
12. Sample size	How many participants were in the study?	20
13. Non-participation	How many people refused to participate or dropped out? Reasons?	6
Setting		
14. Setting of data collection	Where was the data collected? e.g. home, clinic, workplace	Workplace, clinic, office
15. Presence of non-participants	Was anyone else present besides the participants and researchers?	No
16. Description of sample	What are the important characteristics of the sample? e.g. demographic data, date	Interviews conducted Dec 2012-March 2013 Fifteen interviews, were conducted face-to-face and five via telephone; due to geographical or time constraints.
Data collection		
17. Interview guide	Were questions, prompts, guides provided by the authors? Was it pilot tested?	No It was pilot tested
18. Repeat interviews	Were repeat inter views carried out? If yes, how many?	No
19. Audio/visual recording	Did the research use audio or visual recording to collect the data?	Audio recording
20. Field notes	Were field notes made during and/or after the inter view or focus group?	Yes
21. Duration	What was the duration of the inter views or focus group?	The median length of interview ranged from 53 – 56 minutes.
22. Data saturation	Was data saturation discussed?	No
23. Transcripts returned	Were transcripts returned to participants for comment and/or correction?	Yes
Domain 3: analysis and findings		
Data analysis		
24. Number of data coders	How many data coders coded the data?	Two
25. Description of the coding tree	Did authors provide a description of the coding tree?	No
26. Derivation of themes	Were themes identified in advance or derived from the data?	Derived from the data
27. Software	What software, if applicable, was used to manage the data?	NVivo
28. Participant checking	Did participants provide feedback on the	No

	findings?	
Reporting		
29. Quotations presented	Were participant quotations presented to illustrate the themes/findings? Was each quotation identified? e.g. participant number	Yes
30. Data and findings consistent	Was there consistency between the data presented and the findings?	Yes
31. Clarity of major themes	Were major themes clearly presented in the findings?	Yes
32. Clarity of minor themes	Is there a description of diverse cases or discussion of minor themes?	Yes (Presented in Table 2)

Once you have completed this checklist, please save a copy and upload it as part of your submission. When requested to do so as part of the upload process, please select the file type: *Checklist*. You will NOT be able to proceed with submission unless the checklist has been uploaded. Please DO NOT include this checklist as part of the main manuscript document. It must be uploaded as a separate file.

Identifying priority medicines policy issues for New Zealand; a general inductive study.

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Identifying priority medicines policy issues for New Zealand; a general inductive study

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Abstract

Objectives: To identify priority medicines policy issues for New Zealand.
Setting: Stakeholders from a broad range of healthcare and policy institutions including primary and secondary care

Participants: Exploratory, Semi-structured interviews were conducted with 20 Stakeholders, throughout New Zealand.

Primary and secondary outcome measures: The interviews were digitally recorded, transcribed, coded into INVIVO 10, then compared and grouped for similarity of theme. Perceptions, experiences and opinions regarding New Zealand medicines policy issues were recorded.

Results: A large proportion of Stakeholders appeared unaware of New Zealand's medicines policy. In general, the Policy was considered to offer consistency to guide decision making. Concerns raised were; by whom and how decisions are made and whether desired health outcomes are being measured. Other concerns included; inconsistencies in evidence and across health technologies. Despite attempts to enable equitable access to medicines; lower socioeconomic (including rural residents) Māori and Pacific ethnicities and, rare disorders have continued inequitable access based upon need. Māori had the added issue of higher disease burden and the resultant need for an "inequity lens". Other issues related to physical access, convenience to and affordability of prescribers and, the increase of prescription fees from $3 to $5. Concerns related to PHARMAC included; a constraining budget, non-transparency of in-house analysis, lack of consistency in recommendations between the Pharmacology and Therapeutics Advisory Committee (PTAC) and its subcommittees, its future ability to make autonomous decisions and affordability - with respect to both the Trans-Pacific Partnership Agreement (TPPA) and increases in demand and cost of new medicines. Constraints and inefficiencies in the submission process to access High Cost Medicines also exist.
Conclusion: The results suggest equitable ability for the general population to have funded medicines prescribed. However, vulnerable groups and some procedures still continue to have issues, not necessarily as a direct result of Medicines Policy or PHARMAC.

Strength and limitations of the study

Strengths

- This study is the first independent objective study to identify priority medicines policy issues, from a broad range of Stakeholders.
- There was reasonable satisfaction with the New Zealand Medicines Policy and its principles. In particular that provision of medicines is evidence based, cost effective and there is equitable ability to have prescribed medicines listed as funded, on PHARMAC's schedule.
- Some patient groups still experiencing difficulties in access, particularly groups with rare disorder and the low socio economically oriented; including rural, Māori and Pacifica populations.
- Other medicines policy issues include pharmaceutical industry's pricing of new medicines; medicines registration requirements, submission for funding process, , budgetary constraints for

medicines, cultural and health literacy, patient affordability, access to prescribers and the measurement of health outcomes.

Limitations

- The views expressed are from 20 Stakeholders. Issues raised in this research project are therefore indicative. Further research is required to explore the indicative issues.

Introduction

New Zealand has a population of approximately 4.5 million, with a nominal Gross Domestic Product (GDP) of approximately $211 billion.(1) New Zealanders have an average life expectancy of 81.2 years, which is above the OECD average of 80.1years.(2) Just under 83 percent (82.7%) of health expenditure in New Zealand is publicly funded.(3) New Zealand's health and disability budget is $13.983 billion.(3) In comparison to other Organisation for Economic Co-operation and Development (OECD) countries, as a percentage of total expenditure on health, New Zealand spends less on pharmaceuticals.(2) Approximately $795 million and $280 million are available, for procuring community/cancer and hospital pharmaceuticals respectively.(4) This compares with a reported estimated spend of $880 million on medical devices.(5) Approximately 1848 medicines are subsidised by PHARMAC, for use in the community, mostly accessible via prescription from a medical doctor.(6)

Medicines and New Zealand

Medicines make a significant contribution to health outcomes.(7) In 2007, "Medicines New Zealand" New Zealand's medicines policy, was launched in response to access concerns from the public.(7) The aim of the policy is to promote quality, effective and optimally used medicines. To guide decisions, principles of: affordability, equity and need are stated.(7) Medicines New Zealand aims to ensure that the decisions made about prioritisation and funding are as transparent as possible, understood and open to debate. It is important for New Zealanders to have confidence that the medicine system is fair, even if they do not always agree with all of the decisions made (7).

Pharmaceutical Management Agency (PHARMAC)

PHARMAC, established in 1993 in response to increasing expenditure on pharmaceuticals, is a separate non-profit government agency, whose role is to determine and procure, community and oncology medicines on behalf of the District Health Boards (DHBs) PHARMAC has a pre-determined fixed budget which it is required to operate within. In order to provide medicines considered necessary, PHARMAC employ therapeutic and economic analyses to guide decisions. Their scope is now expanding to include hospital medicines and some medical devices. For the majority of patients prescribed a medicine listed on PHARMAC's schedule, a $5 District Health Board charge is incurred. For high user or low socioeconomic patients, access enablers, such as the Community Services and High User cards and, now the Services to Improve Access (SIA) exist to help ease financial burden.(6) Additional sources of government funding include: other government agencies such as (Accident Compensation Corporation (ACC)) local government, private medical cover and patient "out-of-pocket" co-payments. (6)

Literature exist indicating medicines issues for New Zealand related to: inequities in access, affordability, processes used and their funding. (8-31) However, no systematic work has been conducted to identify priority medicines policy issues with regards to access and funding of medicines. Within this context, it was considered timely and appropriate to conduct research that could identify priority medicines policy issues for New Zealand.

The dataset obtained from this project was expected to be substantial and provide a solid platform contributing towards informing: medicines policy, expenditure and provision, including the development of optimal medicines management strategies.

Aim

The aim of this project was to identify priority medicines policy issues for New Zealand.

Methods

Study Design and Participant Selection

We conducted a general inductive study, using semi-structured exploratory interviews during December 2012-March 2013. Selection was purposeful, to ensure a broad representation of stakeholders and their opinions, who had one or more of the following traits in relation to medicines policy: involvement in its formation or implementation, had researched and/or commented on medicines policy, including having made submissions during its development, (n = 10); medically qualified doctor (n = 7, 4 of whom were active prescribers); medicines regulation (n = 1); representation of or, past or current involvement in medicines supply, procurement, funding or provision (excluding dispensing, n = 6); belonging to one of the ethnicities in question (n = 4); involved in medicines management (n = 9); medical information or health technology assessment interest (n = 2); medical interest group representative (past or present, n=4); private health provision and subsidy (n = 1); patient group representative (n=2). The participants characteristics are shown in table one.

A total of 26 stakeholders were contacted and explained the research involvement. Twenty stakeholders consented and interviewed. All 20 received a "Participants Information" letter, detailing the involvement, aim and general methods. All signed a confidentiality and anonymity agreement. Fifteen interviews, were conducted face-to-face and five via telephone, due to geographical or time constraints. The average length of interview ranged from 53 – 56 minutes. No gratuity was offered.

Instrument development

The main aim of this research was to identify priority medicines policy areas. An in-depth literature review was conducted, to ascertain existing information on pharmaceutical policy. A total of 105 references were identified as useful. The following broad themes were discovered and accordingly, sets of questions developed: (1) Medicines Policy: including participant's awareness, description and opinions, (2) Ethnicity inequities in accessing medicines (viz Māori, Pacifica and recently immigrated people whose first language was not English), (3) PHARMAC: its pricing policy, impact upon access, economic modelling, performance, future and any improvements (4) The Trans Pacific Partnership Agreement(TPPA): impact upon access and resultant considerations (5) High cost medicines access (6) Medicines policy issues not covered but considered important (see appendix one for question details)

The questions were piloted on one doctor of Māori ethnicity and one pharmacist with an interest in medicines policy, medicines management and academia, who has previous experience in the pharmaceutical industry. Their responses were not included for analysis.

Data Collection

Participants were encouraged to give comprehensive answers. Clarifying and confirming questions were asked where more information was considered necessary, or to avoid interviewer assumption.

All interviews were recorded on a voice recorder, transcribed intelligently (space fillers were omitted to enable ease of reading) Participants received their own transcript to proof, edit and approve. Only the approved editions were entered into INVIVO 10 (QSR International Pty Ltd) for coding.

Coding was conducted two ways: firstly, categorically according to answers and secondly, highlighted, grouped and compared – according to similarity of theme. Transcripts were checked for any missed issues.

A check for stakeholder bias was conducted using the coding summaries, no apparent bias was detected. Any variations appeared attributable to stakeholder knowledge; so were to be expected.

Results

Issues revealed specific to ethnicity, PHARMAC, the TPPA or high cost medicines are reported in those sections. A summary of issues is available in table two.

General Medicines Policy Issues

Nine participants stated they were unfamiliar with the policy. However, four demonstrated a tacit understanding. It was questioned how policy intentions and decisions are made, in the context of being achievable:

> *"How do you attain that?...what is the right way to make those overall policy decisions…" (PI)*

All participants believed medicines make a positive contribution to health. Differing levels of impact upon health were noted. There was uncertainty as to how the impact is, or could be quantified. The lost opportunity from not capturing and accessing data efficiently, was voiced by two academics for both treatment and outcomes monitoring:

> *"…we are not asking questions about patient health status before and after… so you can really see what is going on, at the GP level. Because that's at least as important as hospitalisation data". (Ac)*

Conversely, one participant said he would prefer to see more investment in epidemiology, as opposed to increasing the medicines budget, in a desire to preserve health.

Low socioeconomic patients were considered to have a higher burden of disease. Affordability to prescribers was described as the major issue, which may be compounded by the 2013 raise in prescription co-payment from $3 to $5. Australia was contrasted, where there are comparatively low prescriber and higher prescription co-payments.

Despite access enablers, such as the High User Cards and Community Services Cards, it was questioned whether those in need are utilising them. One GP said cost-sensitive patients could be managed with prudent prescribing and education on priorities:

> *"You could get all your medicines for less than a pack of cigarettes. It's educational priorities and various other things, where the effort needs to go rather than reducing the cost much further." (GP)*

The opposite situation of the misuse of access enablers was described:

> *"So it's that whole inverse law." (GP);*

> *"I initially struggled to understand how somebody could pull up outside a pharmacy in a Mercedes Benz and… present their scripts for their family and handover their Community Services Card… As soon as they get in the country; they put the money into a family trust...So the wealth of the individual gets assessed, which qualifies them for a Community Services Card and then they wave that around." (Ph)*

Sole supply[1] provision raised issues in terms of: supply outages when switching supplier (and having to pay for the alternative option), options for patient intolerance and vulnerability if a significant disruption in supply occurs (e.g., a disaster destroying a supplier's warehouse).

There was additional reference to policies and funding needing to be consistent and interlink, especially for priority areas. "Quit-Line" was given as an example: a $40 million funded smoking cessation programme, described as having markedly less evidence than the appropriate medicine (which was not funded for many years). Budgetary constraints were the reason given for this. Equally, the government funding of "alternative medicines" was described as needing debate:

> "*… government is providing funding for people to obtain alternative medicines … real debate to be had… money better spent some place else in the healthcare system?*" *(PI)*

One Doctor voiced frustration at PHARMAC's Therapeutic Advisory Committee (PTAC) [2] being *"generalists"* who over-ride the recommendation of their subcommittee. With patient sub-typing and genomic medicine on the horizon, he considered *"generalists"* may not understand what they are assessing and dismiss research, thereby inhibiting access. Access is then through an ability to pay for litigation and decided by a non-medical expert.

With demographic changes increasing demand for healthcare services and a general movement towards increasing costs of new medicines, there was concern for future affordability. Suggestion was funding may move away from being population based, toward funding health outcomes. The biggest concern being: the discovery and affordability of a panacea. An academic suggested: changes in co-payments, taxation or medicines classification status may result. The oncologist had concerns that the lack of research (research being an attracter) being conducted within New Zealand, will compound the low availability of future prescribers. Extending prescribing ability to non-doctors was considered to help. However, for oncology, a medical specialist was considered to still be required to make treatment decisions.

Ethnicity Issues

Most issues presented, related to socio-economic variables and are presented under General Medicines Policy Issues.

Those with poor English speaking skills were described as having access to an English speaking relative or even interpreters if needed:

> *"I think if they can access General Practice or the Hospital system, their access to the medications is just as good as anybody else's. I'm not aware of any specific ethnic problems in accessing our medicines.." (GP)*

One GP felt strongly that Māori and Pacifica access inequities are evidenced by poorer health outcomes. He considers his colleagues are treating everyone the same but with inequitable risk: earlier intervention, improved communication, education and patient engagement are required.

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[1] Sole supply arrangements are likely to be used by PHARMAC in markets where generic competition exists, resulting in there being only one brand of a particular chemical listed. It is possible that PHARMAC would agree preferred supplier status for some chemicals in exchange for price concessions, affecting access to related pharmaceuticals within the same therapeutic group.(32. Pharmaceutical Management Agency. Proposed pricing strategy initiatives - sole supply arrangements. Pharmaceutical Management Agency; 2002 [cited]. Available from: http://www.pharmac.govt.nz/2002/07/19/nhps.pdf.

[2] PTAC is PHARMAC's primary clinical advisory committee. PTAC's role is to provide clinical advice to the Board of PHARMAC.

> *"I think the key issue is the prescribers have a poor understanding of inequalities. Because, the prescribers generally approach things as; I treat everyone the same… they must have an inequity lens on anyone they see… but if the quality of your discussion and the quality in the way in which you prescribed that was poor i.e., you culturally are incompetent and you have a disconnect with the patient…" (GP)*

Other issues related to Asian ethnicities wanting treatment (oncology setting) irrespective of likely outcomes and, the use of alternative treatments e.g., St John's Wort or Vitamin C injections impacting upon medical treatment. One of the doctors had issue with alternative practitioners recommending such treatments as safe and evidence based, upon requesting information to support these treatments, he found the paper to be an out-dated and flawed case study.

Pharmaceutical Management Agency (PHARMAC)

There was general appreciation shown towards PHARMAC's strategy of creating competition in order to achieve a lower purchasing price. This was seen as advantageous for the purchasing of a greater range of medicines, in the context of a fixed budget.

The budget was defined as the threshold for provision, which was considered too small by an academic and PI, causing a focus on cost as the driver of value and provision, thereby contributing toward *"static efficiency"*:

> *"If Pharmac's objective is to stay within budget then it's doing well… improve the health of New Zealanders within a capped pharmaceuticals budget…it's doing moderately well…objective were to improve the health of New Zealanders taking into account the financial constraints of Vote Health…it's doing poorly because it should be fighting for a better share of Vote Health." (Ac)*

A public servant made the following comment: *"You can always achieve more with more."* In terms of a bigger budget but there isn't an analytical framework in place which would define whether the medicines budget receives a fair proportion of "Vote Health."

There was concern: whilst PHARMAC's budget is determined at regular defined intervals, medicines enter the marketplace sporadically, for which funds may not be available.

The private health care provider (PHCP) thought PHARMAC's approach of requiring new and more expensive medicines to be better than standard medicines a: *"completely acceptable approach."* It was suggested by the PHCP and an academic that their approach could be more widely adopted, both overseas and with the expansion of PHARMAC's role to medical devices:

"…the expertise PHARMAC has built up…is something that we could learn from and borrow from, for the wider health sector… I'd like to see them take on medical devices, because that is absolutely scandalous that these products are getting onto the market without being properly evaluated…" (Ac)

There was caution given from one pharmacist that PHARMAC's expansion into hospital medicines (in an acute care setting of moribund disease) may limit choices. Concern was shown for risk, if New Zealand is world-leading in this type of provision.Provision was described as having a utilitarian focus: *"The greatest good for the greatest number" (PS)* and described as being: you get what you need - not what you want. One doctor questioned whether the lost opportunity from not treating someone is being measured. Rare disorder patients were mentioned and are discussed under High Cost Medicines. The distinction was made that

provision of a medicine in a cost effective manner, which PHARMAC achieves, is not the same as delivering healthcare:

> *"I find some of their PR a little bit irritating…bray on about the marvellous healthcare they're delivering… delivering medicines in a cost effective manner but that's not saying it's delivering healthcare…" (PHCP)*

The question of the economic modelling Pharmac undertakes received very favourable comments from 15 of the participants. Three participants were not familiar with economic modelling:

> *"I think it's world leading actually. No one else dares do it. That's the crazy thing. Here we are little old New Zealand and we dare do it." (Ac);*

> *"Well I mean, as a tax payer you could argue that for the majority of the products they get in, they've done a really good job of driving cost out of the system." (PI);*

> *"Technically it's very good. PHARMAC considers clinical effectiveness and cost effectiveness.. they make trade-offs… they look at the QALYs and the number of people affected and how their quality of life will be improved and so on, I think is a very good model." (PS)*

It was suggested that cost-benefit should be a consideration because of valuing the return of an individual back to their normal daily activities, such as what Accident Compensation Corporation does in assessing intervention options.

A pharmaceutical industry representative and academic were concerned the required economic modelling submitted by suppliers is adjusted with unknown *"in-house"* variables, making it hard for suppliers to understand decisions. This was contrasted against Medsafe's practice, where decision modelling is transparent:

> *"Pharmac receives a dossier from the company…Assumptions of statistical models get changed…QALYs get changed…population who will use the product get changed…that should be part of a scientific debate…companies don't know what information is being used to make the decisions on their products…we would like a right of reply to those… It happens with MedSafe…Not as though it could potentially negatively affect evidence based decisions."(PI)*

Delays in the submission process of up to eight years and described as a: *"medicines waiting list,"* were of concern for an academic, pharmaceutical industry representative and patient group representative, who all thought access was related to cost. There was suggestion from one academic to follow Australia's submission process and out-source assessments from independent bodies.

Trans Pacific Partnership Agreement (TPPA)

Very few participants were familiar with the TPPA, some referred to speculation and no facts. One participant refused to give any comments related to the TPPA.

There was acknowledgement that trade deals are complex and often require compromises and trade-offs. New Zealand was referred to as a: *"small country"* and *"we need our trade partners."* There was concern

that already "*big amounts"* are being spent on healthcare and the *"benefits are low"* and, if there is a resultant increase in the cost of medicines, where would resources come from, to off-set any cost increases.

The main issues were: (1) Patent extension, delaying generic entry to market, thereby prolonging a higher cost of provision; (2) Industry influencing supply (described as an issue of sovereignty) may result in quicker access to new medicines but also an increase in public campaigns and appeals processes if PHARMAC's decisions are unpopular with the industry or patient groups; and (3) The call for transparency in PHARMAC's assessment process caused the most concern and confusion. One Academic said he didn't think PHARMAC could be more transparent and that transparency might mean the industry discloses its pricing processes and the results of all clinical trials.

In general, scepticism was voiced as to what the driving force is behind the agreement and what the benefits would be for New Zealand - with the USA being a protected market (heavily subsidised) Australia was described by a pharmaceutical industry representative, as getting *"trounced"* over their agreement with the USA, losing a lot of their pharmaceutical production and jobs as a result:

> *"Forget it…wouldn't even bother going along to the negotiations"(PHCP)*;
>
> *"…tell the US to bugger off quite frankly. You either put everything on the table and we talk about it or no, you don't…We should learn from what happened in Australia…" (PI)*

Conversely, another participant suggested whilst *"America"* has influence, it may become limited as a result of the influence of China's developing economy and differing ideas around protection and, new opportunities may develop:

> *"…a hugely developing economy in the form of China that basically has total disregard for such things… so the ability for America…is probably going to be limited in the world of the future, and maybe different forms of protection of ideas will kind of evolve… it's very hard to predict how the market might respond or what kind of new opportunities develop." (PS)*

One academic suggested that PHARMAC's monopsony is an anathema to the USA. A pharmaceutical industry representative said Medicines New Zealand (New Zealand's prescription medicines representative association, same title as the policy) is attempting to ensure its USA equivalent understands New Zealand's medicines system:

> "*…working quite hard to ensure…our sister organisation in the US is effectively asking the US government to achieve out of the process, is well enough informed to understand actually what the New Zealand model does achieve, what it doesn't achieve and how that can be improved…So we're working hard to make sure it's a process that actually benefits New Zealanders as well, and all of the transparency, timeliness, appeals – those aspects that we've discussed, are exactly I think what the US is likely to be asking for.*

Most considered that New Zealand's current ability to access generic medicines or, independence in procuring medicines should be upheld. If not, funds may need to be redirected from other services or, patient co-payments would need to rise, in order to compensate a likely increase in the cost of medicines.

High Cost Medicines

A GP questioned the necessity of continuing the Special Authority (SA)[3] status for a medicine, once the appropriate use of a medicine has been established. Not all participants were familiar with the Named Patient Pharmaceutical Assessment (NPPA)[4] access scheme. Most high cost medicines were described as being *"breakthrough"* or *"expensive"* and are restricted, to control spending. One participant said, if it was *"dirt cheap,"* there would be "no argument," indicating the case even if the medicine didn't have clear health benefits:

> *"My bet, is that PHARMAC would listen to anyone that agrees with them saying no. Because it's expensive ....They are diametrically opposed for a reason and the reason is cost." (Ph);*
>
> *"The Rabbits in charge of the lettuce patch" (M)*

One public servant thought there to be no inappropriate blocking of access to medicines, as no complaints about access have been received at their level. Equally, another Public Servant commented that there are patients accessing medication costing up to $500, 000 per year:

> *"…So it's not that the system can't cope with treatments that are high cost, it's just that we would expect a return for that cost and for it to be justifiable in terms of what we value." (PS)*

A small group of patients were described as not having access to high cost medicines. Access was described as *"the collective good."* Conversely, *"people dying from a lack of access to very cheap and simple therapies"* were described. It was suggested that it is a DHB's remit to look after its population, highlighting the issue of population versus individual access. A statement was made: are we advocating treatment at any cost and if so, who pays:

> *"It's a question of who pays for all these things. I think if you have pretence; like there is in the USA, that cost isn't of any relevance… then you're going down the wrong path." (PHCP)*

The oncologist described the NPPA process as inefficient: a comprehensive and referenced application, takes him up to 6 hours, potentially impacting on his clinic time and perversely hindering patient access. He suggested PHARMAC at a nominal cost could employ someone to aid in information gathering and in the process develop expertise.

Additionally, the oncologist believes oncology has the stigma that everyone dies but individual survival may be greater than the median survival assessment. This issue was presented in comparing the availability of 2-3 drugs in Australia - unavailable in New Zealand.

Questions were posed: (1) Is it fair to give 4th or 5th line chemotherapy and not give a first line treatment e.g. for rare disorders? and, (2) When do you stop treatment, a patient was described as gaining access to expensive medication, their condition was fragile and they died a few weeks later:

> *"I think it's important if Pharmac has a few loose strings in terms of hospital and severe rare conditions. They are perhaps because of how they are funded, they want a very narrow perspective on those, to try and avoid blow out. They are very emotive issues we don't always know how to best manage people's care. "* (Ph)

[3] Special Authority criteria define the clinical circumstances of patients who can receive funding for the medicine. People may first be required to try a less expensive medicine or the medicine may need to be prescribed by a particular type of health practitioner.

[4] NPPA is a mechanism to give individual named patients access to medicines they need, but which aren't funded on the Pharmaceutical Schedule. NPPA replaces the three Exceptional Circumstances (EC) schemes that PHARMAC previously managed.

High need patients, such as rare disorder patients received the most sympathy for difficulty in access because of the exclusion criteria. Evidence requirements were described as difficult to attain due to low patient numbers. Conversely, the PHCP suggested the supplier needs to produce quality evidence:

> *"…where there's some evidence, but not solid or quality evidence: … the company doing the – providing the medication, it behoves on them to do some research in those areas and produce quality data."(PHCP)*

Discussion

We purposefully attempted to be open to issues and their capture, despite some issues already being identified. Our focus was on access to medicines. It is possible there are other issues in existence, we neither recognised, nor captured. We did not seek to determine issues specifically related to generic medicines, considered a "*vital component of New Zealand's medicine cost management policies*" by Babar et al.(12)

Medicines Policy

Medicines are clearly valued health interventions: evidenced by the budget, literature and responses from stakeholders. The smaller percentage spend on pharmaceuticals in New Zealand (described as a constrained budget) compared with similar countries such as Australia, UK and USA,(2) may in fact reflect the price reduction strategies that are implemented by PHARMAC(32), as opposed to less opportunity to improve health outcomes. However, this needs to be tested through robust research on health outcomes and their relationship to pharmaceutical spending.

Delayed access and the resultant impact discussed by some of the participants, was also described by Ellis and Hamer,(33) in relation to New Zealand's statin availability for atherosclerotic patients, as probably negatively impacting health outcome and considered to be due to the capped budget. They considered this *"anomalous,"* as other types of health care are not capped. This anomaly was also described by a number of participants but may change with PHARMAC's expanding role.

New medicines are increasing in costs along with demand, causing tension in affordability. Price efficiency initiatives, such as what PHARMAC encourages, help ease the tension in affordability of provision. Another option is to reduce demand: either through gate keeping (not usually a popular choice) or genuine effects, such as initiatives to maintain health or prevent disease. We assume a reduction in demand and therefore burden of provision, should result in healthcare becoming more affordable, for providers and helping those remaining in need.

. Manning,(34) compared the processes of: decisions, pricing, economic analysis, provision and access and, participation and appeals between the UK, Australia and New Zealand. It was suggested that resolving issues may benefit from a disputes panel comprising a broad range of experts in: scientific, economic, policy and ethical evaluations, in order to provide an objective decision. Manning additionally reported that approximately one sixth of the United Kingdom's National Institute for Healthcare and Clinical Excellence (NICE) recommendations are appealed and upheld. (35) There shouldn't be great demand, if evaluation processes are robust.

Ethnicity

Our participants revealed that low socioeconomically related populations (encompassing Māori and Pacifica people) are continuing to have access issues related to financial, structural, educational and cultural barriers. These findings were consistent with that of Jatrana et al,(36) who assessed SOFIE-health's 18320 respondents (an add-on to Statistics New Zealand's longitudinal survey of Family, Income and Employment) Māori and Pacific people were more likely to defer purchasing a prescription due to cost, which at that time was $15.

Māori represent approximately 15 percent of New Zealand's population(37) and on average have the poorest health status of any ethnic group in New Zealand.(38, 39) Pacifica people represent 6.5 percent of the population and also experience health inequalities. He Korowai Oranga: the Māori Health Strategy (2002),(38) recognises the Treaty of Waitangi principles of: partnership, participation and protection, through which, the aim is to reduce existing health inequalities. This aim is extended to include the Pacific people, like Māori, they are over represented by a low socioeconomic situation, reflecting low affordability and health literacy, which in turn affects access. The Ministry of Health recently launched Services to Improve Access(SIA),(40) an additional targeted capitation payment, available to Primary Healthcare Organisations(PHOs) to reduce health inequalities. It is designed for new services (e.g., outreach programmes) or improving access (e.g., funding transport) for Māori, Pacific people and those of low socioeconomic status. Once SIA is embedded, it would be prudent to evaluate its impact.

Other Ministry of Health initiatives, such as Whanau Ora (to build the health, participation and capability of families) and, One Heart Many Lives (to improve the cardiac health of Māori and Pacific men) along with recent changes in health practitioner training, appear good initiatives for engaging Māori and Pacifica in a culturally appropriate way. It would be prudent to evaluate their impact.

We did not have any issues specifically described for new immigrants. It was described to us that patients with poor English speaking capability, present to practitioners with an English speaking person, or frequent a surgery of their ethnicity. This is at odds with Babar et al,(9) who found for 11 Chinese and Indian migrants, residing in New Zealand for less than five years, financial barriers existed in affording doctors, pharmacists and medicines and, that language barriers exist. This anomaly may highlight the differences in both the perspective and experience of the stakeholders we interviewed.

Asia and India have different medicines access systems to New Zealand. Babar additionally found there is a lack of information on New Zealand's medicines system, provision and classifications. The United Nations and World Health Organisations, when discussing the right to health,(41) refer to migrants as being vulnerable to reduced access to health services for reasons that include: language or cultural barriers. New Zealand has a significant migrant population, reported as 927 000 in 2006.(42) The current main countries for immigration are: China (15%), United Kingdom (unspecified), India (13%) and the Philippines (8%).(42) In consideration of Babar et al's work and immigration statistics, there may be a significant number of people from these countries with issues, resulting in difficulties in accessing healthcare and therefore medicines.(8-10) In light of this, it may be worth further investigating new immigrant issues.PHARMAC

The general appreciation for New Zealand's need to be efficient to provide more medicines expressed by our stakeholders, was also shown by Ragupathy et al.(26) Included, was the need to apply consistent economic evaluations to other health technologies, to support congruous decisions for resource allocation. PHARMAC's expansion into procuring hospital medicines and medical devices may enable greater consistency of evaluation across technologies.

The significance of PHARMAC's role expansion should not be underestimated. PHARMAC will need to practice caution with expanding their role into hospitals, which are generally settings of acute and moribund disease. We are unaware whether a closed formulary has occurred elsewhere in the world. PHARMAC have been noted to have consulted directly with medical specialist groups to discuss their role expansion, including consultation on hospital medical devices,(43) so would appear to be fully cognisant and appreciative of this issue.

We found delays of up to eight years, in PHARMAC's process for funding medicines onto the Pharmaceutical Schedule, which our stakeholders purported to be due to the medicine's pricing and/or PHARMAC's budget not being able to expand. Other reasons may be the medicine's priority status, insufficient information or, not meeting PHARMAC's nine decision criteria.(44) The question is whether this means delays in therapeutic advancement and therefore improved health outcomes.

The measurement of opportunity foregone was of clear concern to the Oncologist we interviewed. New Zealand has a capped medicines budget, it cannot expand and therefore drives the need for efficient spending (determined using cost utility analysis, where medicines are assessed against QALY gains per $1 million) Using this process for provision means there is opportunity foregone, as described by Milne and Wonder.(14) We are not aware of New Zealand focused research assessing either opportunities foregone or, other specialist viewpoints on access. The exceptions being: Ellis and Hamer(33) in 2008, discussing the delayed availability of cardiac medicines; MacCormack et al in 2009,(31) assessing stakeholders views on needed access to high cost medicines and; The Sage report for the Ministry of Health in 2010, (22) reporting the consultation of stakeholders on the proposal to expand PHARMAC's role.

Sole supply issues (supply outages, lack of palatable formulations, resultant out of pocket payments for alternatives and vulnerability as a result of a disaster) were reported as still continuing, despite there being penalties for suppliers. This was also reported by Babar et al,(45) who reported additional concerns with poor quality products in the past, from previous studies.

Trans-Pacific Partnership Agreement (TPPA)

There is very little information available on the TPPA, the reason given: particulars of the negotiation are changing. What does exist concurs with our findings: it questions the motivation and self-interest of parties involved and warns of possibly binding impacts that may affect health services budgets and PHARMAC's autonomy, including method of procurement and provision.(25-29, 46) Such impacts stem from the USA's desire for stricter protection of intellectual property rights, transparency of in-house evaluation, regulatory coherence, dispute settlement, government procurement and evidence based decisions being contestable in court. Unless budgets are expanded to cope with likely increases in costs, there may need to be a re-evaluation of provision, subsidies and co-payments. In contrast to existing publications, our research additionally suggested a TPPA may enable earlier access to newer medicines. It may be of use to quantify what effect a TPPA would have upon medicines access.

High Cost Medicines

A significant issue discussed in our study, was the need to differentiate between high cost medicines and highly specialised needs or medicines in relation to the NPPA access scheme. McCormack et al, (31) suggest a medicine that costs $20 000 per patient per year may be considered high cost. It is important to be

cognisant of the total cost to the health system of any medicine, which is dependent upon the number of patients treated (volume used) and the acquisition price. Some high cost medicines may not result in a high total cost to PHARMAC, for some patient groups. Gallego et al,(47) question how treating large populations at high total cost for small population gains, compares with treating smaller populations, for possibly significant benefit.

The issue of treating large versus small populations may intensify with patient subtyping and genomic medicines development (as described by the oncologist) where greater expectation to fund (i.e., demand) may occur. With the NPPA process now reported in our findings, as enabling the capturing of cancer patient subtype information, cancer medicines outcomes may become easier to measure and if positive, make it harder to decline funding treatments. It may also mean the table is turned and large populations end up having limited treatment options, if outcomes cannot be measured in the same way. However, funding outcomes will give a clear indication for innovation and direction to both suppliers and funders of medicines.

Our findings describe both the SA and the NPPA access schemes as being inefficient. The SA inefficiency finding is also supported by Babar et al's evaluation of GP perceptions on access to medicines in New Zealand.(45) Once correct prescribing of a medicine has been established it may not be necessary to continue a medicine's SA status. The NPPA process appears to impact significantly on consultant clinic time which may perversely hinder patient access. With demographic trends indicating greater demand for such medicines, the impact of the inefficiency may intensify. PHARMAC's website lists 555 approvals and 15 declines for NPPA access.(48) It may be more efficient for PHARMAC, at a nominal cost, to contract an evidence based facilitator, to ease the burden of application for clinicians.

Difficulty in access to high cost medicines, encompassing rare disorder patients, as described by our participants, has been widely documented.(31, 47, 49-58) The nature of rare diseases makes it hard to gain the necessary evidence PHARMAC requires for evaluation. This issue is compounded for suppliers because the need to satisfy both manufacturer ordering and regulatory requirements, adds to the unit cost of supply for low volume demand medicines. It may be worth investigating options to reduce cost of supply and provision in the context of constrained evidence. PHARMAC have recently sought public and professional input into its decision criteria. The results have yet to be published but may reveal new options or initiatives.

Our research highlighted the issue of access to medicines of therapeutic value in the context of a fixed predetermined budget and the difficulties in how priorities for funding are determined. Lu et al,(59) in discussing ethical perspectives to the access of high cost medicines in Australia, discussed the issue of having equal need requires equal opportunity to access care and suggest where evidence requirements are not achieved, treatment commence on a trial and outcome basis. This does come with ethical concerns but may enable both access and capturing evidence. MacCormack et al,(31) suggest "risk sharing" supply to ensure some form of access (defining a threshold for maximum numbers to treat for a high cost medicine, above which, the supplier funds)

Conversely, Simoens et al,(52) caution providing access to medicines with limited effectiveness, implies rare disorders health improvement is more valuable than a common disease, which challenges the utilitarian view of: the health gain of each patient is valued equally. With both increasing effort in the development and availability of orphan drugs, this issue may only worsen. Equally, other questions arise: because we see the ill health, can't mean preferential treatment over someone who has a "silent" state of declining health. There are people not getting access to inexpensive medicines, who are at risk, as stated by an academic. Perhaps remedying issues of access based on need, could start with prioritising based on the impact of an unmet need?

Conclusion

Overall, despite issues being identified, there was reasonable satisfaction with the New Zealand Medicines Policy and its principles. In particular that provision is evidence based, cost effective and there is equitable ability to have prescribed medicines listed as funded, on PHARMAC's schedule.

However, despite this, there appears to be some patient groups still experiencing difficulties in access, not necessarily appearing as a result of medicines policy or PHARMAC. Such groups being: rare disorders and the low socio economic (encompassing rural, Māori and Pacifica populations) Other issues ranged from: the pharmaceutical industry's pricing of new medicines, manufacturer and registration requirements, the submission for funding process, increasing demand and costs, budgetary constraints, cultural and health literacy, patient affordability and access to prescribers, through to knowledge development for clinical expertise and the measurement of health outcomes.

Our study has highlighted issues in access based upon need and the consequences of unmet need. The context being: a fixed, predetermined budget and increasing demand, is causing constraints in affordability. We suggest these issues and consequences of unmet needs may worsen and options for demand and provision may need to be explored further.

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Authors contribution: ZB conceptualised and designed the study. The data collection, entry and analysis was handled by SF and ZB. SF and ZB wrote the manuscript. The final version is approved by all authors. ZB acts as an overall guarantor to this study.

Transparency: ZB affirms that the manuscript is an honest, accurate, and transparent account of the study being reported; that no important aspects of the study have been omitted.

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References

1. Statistics New Zealand. Top Statistics. 2013 [cited]. Available from: http://www.stats.govt.nz/browse_for_stats/snapshots-of-nz/top-statistics.aspx.
2. Organisation for Economic Co-operation and Development countries. OECD Health Data 2013 - frequently requested data: health expenditure. 2013 [cited]. Available from: http://www.oecd.org/els/health-systems/oecdhealthdata2013-frequentlyrequesteddata.htm.
3. Ministry of Health. Funding. Ministry of Health; 2013 [cited 2013]. Available from: http://www.health.govt.nz/new-zealand-health-system/funding.
4. Pharmaceutical Management Agency. Setting and managaing the combined pharmaceutical budget (CPB). New Zealand Government; 2013 [updated 2013; cited 2013]. Available from: http://www.pharmac.health.nz/ckeditor_assets/attachments/381/infosheet-08-community-pharm-budget-2013.pdf.
5. Pharmaceutical Management Agency. PHARMAC and hospital medical devices. 2013 [cited]. Available from: http://www.pharmac.health.nz/medicines/hospital-devices/.
6. Pharmaceutical Management Agency. New Zealand Pharmaceutical Schedule. 2013 [updated April, 2013; cited 2013]. Available from: http://www.pharmac.govt.nz/2013/04/03/Sched.pdf.
7. Associate Minister of Health, Minister of Health. Medicines New Zealand: Contributing to good health outcome for all New Zealanders. In: Health Mo, editor. Wellington, New Zealand: Ministry of Health, Wellington, New Zealand; 2007.
8. DeSouza R, Garret N. Access Issues for Chinese People in New Zealand Final Report. : Auckland University of Technology, Auckland; 2005 2005. Contract.
9. Babar ZUD, Pengelly K, Scahill SL, et al. Migrant health in New Zealand: Exploring issues concerning medicines access and use. Journal of Pharmaceutical Health Services Research. 2013;4(1):41-9.
10. Suin T. An holistic approach to Asian health Fourth internaitonal Asian health and well-being conference; 2010; Auckland. 2010.
11. Ragupathy R, Aaltonen K, Tordoff J, et al. A 3-dimensional view of access to licensed and subsidized medicines under single-payer systems in the US, the UK, Australia and New Zealand. PharmacoEconomics. 2012;30(11):1051-65.
12. Babar ZUD, Grover P, Stewart J, et al. Evaluating pharmacists' views, knowledge, and perception regarding generic medicines in New Zealand. Research in Social and Administrative Pharmacy. 2011;7(3):294-305.
13. Wonder M, Milne R. Access to new medicines in New Zealand compared to Australia. New Zealand Medical Journal. 2011;124(1346):12-28.
14. Milne R, Wonder M. Response to PHARMAC on access to new medicines in New Zealand compared to Australia. New Zealand Medical Journal. 2011;124(1347):18.
15. Tordoff JM, Norris PT, Reith DM. "Price management" and its impact on hospital pharmaceutical expenditure and the availability of medicines in New Zealand hospitals. Value in Health. 2008;11(7):1214-26.
16. Aaltonen K, Ragupathy R, Tordoff J, et al. The impact of pharmaceutical cost containment policies on the range of medicines available and subsidized in Finland and New Zealand. Value in Health. 2010;13(1):148-56.
17. Cumming J, Mays N, Daubé J. How New Zealand has contained expenditure on drugs. BMJ (Online). 2010;340(7758):1224-7.
18. Metcalfe S, Grocott R. Comments on "simoens, s. health economic assessment: A methodological primer. int. j. environ. res. public health 2009, 6, 2950-2966"-New Zealand in fact has no cost-effectiveness threshold. International Journal of Environmental Research and Public Health. 2010;7(4):1831-4.
19. Moodie P, Metcalfe S, Poynton M. Do pharmaceutical score cards give us the answers we seek? New Zealand Medical Journal. 2011;124(1346):69-74.
20. Moodie P, Metcalfe S, McNee W. Response from PHARMAC: Difficult choices. New Zealand Medical Journal. 2003;116(1170).
21. Grocott R. Applying Programme Budgeting Marginal Analysis in the health sector: 12 years of experience. Expert Review of Pharmacoeconomics and Outcomes Research. 2009;9(2):181-7.
22. Sage D. Report on the consultation period for the proposal to expand the functions of PHARMAC; 2010. Contract.
23. Morgan S, Boothe K. Prescription drug subsidies in Australia and New Zealand. Australian Prescriber. 2010;33(1):2-4.

24. Carlson JJ, Sullivan SD, Garrison LP, et al. Linking payment to health outcomes: A taxonomy and examination of performance-based reimbursement schemes between healthcare payers and manufacturers. Health Policy. 2010;96(3):179-90.
25. Gleeson D, Lopert R, Reid P. How the Trans Pacific Partnership Agreement could undermine PHARMAC and threaten access to affordable medicines and health equity in New Zealand. Health Policy. 2013.
26. Ragupathy R, Tordoff J, Norris P, et al. Key informants' perceptions of how pharmac operates in New Zealand. International Journal of Technology Assessment in Health Care. 2012;28(4):367-73.
27. Moynihan R. New Zealand agency comes under pressure to pay more for drugs. BMJ (Clinical research ed). 2011;342.
28. Foster RH, Wilson N. Trans-pacific partnership: Will Pharmac become a victim of its own success? BMJ (Online). 2011;343(7818).
29. Faunce TA, Townsend R. The Trans-Pacific Partnership Agreement: challenges for Australian health and medicine policies. Med J Aust. 2011;194(2):83-6.
30. Raftery JP. Paying for costly pharmaceuticals: regulation of new drugs in Australia, England and New Zealand. Med J Aust. 2008;188(1):26-8.
31. MacCormack P, Quigley J, Hansen P. Review of access to high-cost, highly specialised medicines in New Zealand. ; 2010. Contract.
32. Pharmaceutical Management Agency. Proposed pricing strategy initiatives - sole supply arrangements. Pharmaceutical Management Agency; 2002 [cited]. Available from: http://www.pharmac.govt.nz/2002/07/19/nhps.pdf.
33. Ellis CJ, Hamer AW. Cardiovascular health in New Zealand: areas of concern and targets for improvement in 2008 and beyond. New Zealand Medical Journal. 2008;121(1269):5-10.
34. Manning J. Priority-setting processes for medicines: the United Kingdom, Australia and New Zealand. J Law Med. 2011;18(3):439-52.
35. National Institute for Health and Care Excellence, . [cited]. Available from: http://www.nice.org.uk.
36. Jatrana S, Crampton P, Norris P. Ethnic differences in access to prescription medication because of cost in New Zealand. Journal of Epidemiology and Community Health. 2011;65(5):454-60.
37. Statistics New Zealand. Māori population estimates. 2012 [cited]. Available from: http://www.stats.govt.nz/browse_for_stats/population/estimates_and_projections/maori-population-estimates.aspx.
38. Ministry of Health NZG. He Korowai Oranga: Māori Health Strategy, . 2002 [cited]. Available from: http://www.health.govt.nz/publication/he-korowai-oranga-maori-health-strategy.
39. Ministry of Health NZG. The Health of Māori Adults and Children. 2013 [cited]. Available from: http://www.health.govt.nz/publication/health-maori-adults-and-children.
40. Ministry of Health NZG. Services to improve access. 2011 [cited]. Available from: http://www.health.govt.nz/our-work/primary-health-care/primary-health-care-services-and-projects/services-improve-access.
41. The United Nations WHO. The right to health. 2008 [cited]. Available from: http://www.who.int/hhr/activities/Right_to_Health_factsheet31.pdf.
42. Ministry of Business Innovation and Employment. New Zealand Government. Migration research. [cited]. Available from: http://dol.govt.nz/research/migration/monthly-migration-trends/13jun/index.asp.
43. Pharmaceutical Management Agency. Adapting the PHARMAC model for medical devices management,. 2013.
44. Pharmaceutical Management Agency. Decision criteria Pharmac; 2013 [cited 2013 11.4.13]. Available from: http://www.pharmac.health.nz/medicines/how-medicines-are-funded/decision-criteria.
45. Babar ZUD, Grover P, Butler R, et al. A qualitative evaluation of general practitioners' perceptions regarding access to medicines in New Zealand. BMJ Open. 2012;2(2).
46. Kelsey J. Hidden agendas what we need to know about the TPPA. BWB texts; 2013 [cited. Available from: www.bwb.co.nz.

47. Gallego G, Taylor SJ, Brien JaE. Funding and access to high cost medicines in public hospitals in Australia: Decision-makers' perspectives. Health Policy. 2009;92(1):27-34.
48. Pharmaceutical Management Agency. NPPA outcome report cumulative for March - December (updated 24/1/13). Pharmaceutical Management Agency,; 2013 [cited]. Available from: http://www.pharmac.health.nz/ckeditor_assets/attachments/249/summary_of_nppa_decisions_dec_12.pdf.
49. Forman J, Taruscio D, Llera VA, Barrera LA, Coté TR, Edfjäll C, et al. The need for worldwide policy and action plans for rare diseases. Acta Paediatrica, International Journal of Paediatrics. 2012;101(8):805-7.
50. Lu CY, Williams K, Day R, et al.Access to high cost drugs in Australia. British Medical Journal. 2004;329(7463):415-6.
51. Lee TH, Emanuel EJ. Tier 4 drugs and the fraying of the social compact. New England Journal of Medicine. 2008;359(4):333-5.
52. Simoens S, Cassiman D, Dooms M, Picavet E. Orphan drugs for rare diseases: Is it time to revisit their special market access status? Drugs. 2012;72(11):1437-43.
53. Munoz J, Kurzrock R. Targeted therapy in rare cancers-adopting the orphans. Nature Reviews Clinical Oncology. 2012;9(11):631-42.
54. Hudson I, Breckenridge A. The challenges of orphan drugs and orphan diseases: Real and imagined. Clinical Pharmacology and Therapeutics. 2012;92(2):151-3.
55. Drummond MF, Wilson DA, Kanavos P, et al. Assessing the economic challenges posed by orphan drugs. International Journal of Technology Assessment in Health Care. 2007;23(1):36-42.
56. Joppi R, Bertele V, Garattini S. Orphan drugs, orphan diseases. The first decade of orphan drug legislation in the EU. European Journal of Clinical Pharmacology. 2013;69(4):1009-24.
57. Largent EA, Pearson SD. Which orphans will find a home? The rule of rescue in resource allocation for rare diseases. Hastings Center Report. 2012;42(1):27-34.
58. Cheema PK, Gavura S, Migus M, et al. International variability in the reimbursement of cancer drugs by publically funded drug programs. Current Oncology. 2012;19(3):e165-e76.
59. Lu CY, Macneill P, Williams K, et al. Access to high cost medicines in Australia: Ethical perspectives. Australia and New Zealand Health Policy. 2008;5.

Table one: Stakeholder Characteristics:

Stakeholder Group	Number (n)	Professional Title/Characteristics (n)
Academia (Ac)	3	Sociologist (2) Pharmacoeconomist (1)
Public Service (PS)	5	Politician (1) Medsafe (1) Policy Analyst (1) DHB Planning (1) Pharmac (1)
Medicine (M)	4	Oncologist (1) General Practice (3)
Pharmacist (Ph)	3	DHB (2) Community based (1)
Pharmaceutical Industry (PI)	2	Manufacturing (1) Representative (1)
Patient Group Representative (PGR)	2	Long Term Conditions (2)
Private Health Care Organisation (PHCO)	1	Medical doctor (1)

Table 2: Summary of issues

General Medicines Policy	Ethnicity	PHARMAC	TPP	HCM
• By whom and how are decisions made? • Poor Medicines Policy awareness • Poor Health Literacy; impacting timing of presentation and medicines adherence • Access to Prescribers; physical, timing and affordability • Socio-economic factors (encompassing rural residents) • Sole Supply; out of stock vulnerability and cost, options for intolerance • Discord in recommendations between PTAC and subcommittees • Access challenges on the ability to pay for litigation; non-medical person then decides access • Lack of health impact monitoring • Need for integrated electronic patient records, prescribing information & PHARMAC schedule • Efficiency is static; needs to move toward increases in therapeutic benefit • Registration, evidence and manufacturing requirements constraining for low demand medicines • Increasing demand and cost of medicines impacting affordability • Need for clinical expertise and New Zealand specific research • Need for better medicines	• Socioeconomic factors • Need to use "Health Equity Assessment Tool" to assess policy & inequities/inequalities • Higher burden of disease for Māori and Pacifica; needing risk factor lens • Lacking proper engagement at times • Cultural competency • Use of alternative medicine • Need to capture ethnicity statistics in new initiatives	• Very powerful position of provision; will they cope with role expansion • Young inexperienced staff and high attrition rate • What health outcomes are being measured • Is the lost health opportunity being measured? • Budget too small; need higher percentage of Health budget; "Vote Health" • Cost driving value & causing delays • Need to move to dynamic efficiency • Need analytical framework to compare all health technologies • Submission process inefficient • Economic evaluation influencing therapeutic value evaluation; need to be separated • Questionable how well health professionals understand pharmacoeconomic modelling • In-house economic variables are not necessarily consistent with standard practice or PHARMAC's requirements of suppliers • Hard for suppliers to understand outcome or evaluation process when variables changed • Website very informative but hard to navigate • Concern with expansion into hospitals & limiting choice in acute care & moribund disease setting • Sustainability of current access with	• Many unfamiliar and sceptical of the benefits and who gets them vs. the trade offs • New Zealand small country that needs trade partners • Where will the financial cost be felt and how will it be dealt with • Will there be an increase in the cost of provision • A lot money being spent on health already and benefits low • Australia lost a lot with their agreement with USA; we should learn from it • America's influence is reducing and other forms or protection may evolve • Patent extensions will delay generic entry and raise costs • Will the pharmaceutical industry have greater influence on supply • Access to new medicines may improve • Sovereignty of choice; will there be increased public appeals & litigation • What does transparency mean and does it "cut both ways" • PHARMAC's monopsony is an anathema to the USA • NZ pharmaceutical representative educating "sister" organisation in NZ system • Once a medicine is registered for use, it can be prescribed; PHARMAC may choose to not	• Special Authority access unnecessary once appropriate prescribing established • Need to differentiate high cost vs. highly specialised need and cost • NPPA access scheme brings equitable access for oncology but too early to assess • NPPA capturing patient sub-type classification • NPPA process inefficient and consuming valuable specialist time • Limits access due to cost; but about collective good and who pays • Access cheaper in other countries? • Pharmaceutical companies have good profit margins • Oncology stigma that everyone dies but differences in survival seen at the margins • Evidence does not meet PHARMAC's evaluation criteria • Constraints of "rule of rescue" vs. utilitarian provision

management		increasing demand • Affordability of a panacea	fund it	

~~Title~~

~~Identifying priority medicines policy issues for New Zealand; a general inductive study.~~

Identifying priority medicines policy issues for New Zealand; a general inductive study.

Strength and limitations of the study

Strengths

- This study is the first independent objective study to identify priority medicines policy issues, from a broad range of Stakeholders.
- There was reasonable satisfaction with the New Zealand Medicines Policy and its principles. In particular that provision of medicines is evidence based, cost effective and there is equitable ability to have prescribed medicines listed as funded, on PHARMAC's schedule.
- Some patient groups still experiencing difficulties in access, particularly groups with rare disorder and the low socio economically oriented; including rural, Māori and Pacifica populations.
- Other medicines policy issues include pharmaceutical industry's pricing of new medicines; medicines registration requirements, submission for funding process, , budgetary constraints for medicines, cultural and health literacy, patient affordability, access to prescribers and the measurement of health outcomes.

Limitations

- The views expressed are from 20 Stakeholders. Issues raised in this research project are therefore indicative. Further research is required to explore the indicative issues.

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ABSTRACT

Objectives: To identify priority medicines policy issues for New Zealand.

Setting: Stakeholders from a broad range of healthcare and policy institutions, including primary and tertiary care

Participants and Design: Exploratory, semi-structured interviews were conducted with 20 stakeholders, throughout New Zealand.

Methods and measures: The interviews were digitally recorded, transcribed, coded into INVIVO 10, then compared and grouped for similarity of theme. Perceptions, experiences and opinions regarding New Zealand's medicines policy issues were recorded.

Results: A large proportion of stakeholders appeared unaware of New Zealand's medicines policy. In general, the policy was considered to offer consistency to guide decision making. With consideration of fixed budget provision, there was reasonable satisfaction that a subsidised medicine was available for most conditions, rare disorders being the exception. Concerns raised were: by whom and how decisions are made and whether desired health outcomes are being measured. Other concerns included inconsistencies in evidence and across health technologies. Despite attempts to enable equitable access to medicines, lower socioeconomic (including rural residents, Māori and Pacific ethnicities) and, rare disorders have continued inequitable access based upon need. Māori had the added issue of a higher disease burden and the resultant need for an "inequity lens." Other issues related to physical access, convenience to and

affordability of prescribers and, the increase of prescription fees from $3 to $5. Concerns related to the Pharmaceuticals Management Agency (PHARMAC) included: a constraining budget, non-transparency of in-house analysis, lack of consistency in recommendations between the Pharmacology and Therapeutics Advisory Committee (PTAC) and its subcommittees, its future ability to make autonomous decisions and affordability, with respect to both the Trans-Pacific Partnership Agreement (TPPA) and, increases in demand for and the cost of new medicines. Constraints and inefficiencies in the submission process to access high-cost medicines also exist.

Conclusion: The results suggest that overall, there is reasonable satisfaction with the availability of subsidised medicines in New Zealand. However, difficulty in accessing medicines continues: due to socioeconomic factors, evidence requirements for funding, the funding assessment process and the rising cost of new medicines.

Strength and limitations of the study

Strengths

- This study is the first independent objective study to identify priority medicines policy issues, from a broad range of stakeholders.
- Has identified access to medicines issues with which to conduct further research.
- Provides a context to issues.

Limitations

- The views expressed are from 20 stakeholders. Issues raised in this research project are therefore indicative. Further research is required to explore these issues.

Introduction

New Zealand has a population of approximately 4.5 million, with a nominal Gross Domestic Product (GDP) of approximately $211 billion.(1) ~~Just under 83 percent (82.7%) of health expenditure is publicly funded for those eligible.(2) New Zealand's health and disability budget is $13.983 billion.(3)~~ New Zealanders have an average life expectancy of 81.2 years, which is above the OECD average of 80.1years.(2) ~~In comparison to other Organisation for Economic Co-operation and Development (OECD) countries, as a percentage of total expenditure on health, New Zealand spends less on pharmaceuticals. (2) New Zealanders have an average life expectancy of 81.2 years,(2) which is above the OECD average of 80.1years.(2)~~ Just under 83 percent (82.7%) of health expenditure in New Zealand is publicly funded.(3) New Zealand's health and disability budget is $13.983 billion.(3) In comparison to other Organisation for Economic Co-operation and Development (OECD) countries, as a percentage of total expenditure on health, New Zealand spends less on pharmaceuticals.(2) Approximately $795 million and $280 million are available, for procuring community/cancer and hospital pharmaceuticals respectively.(4) This compares with a reported estimated spend of $880 million on medical devices.(5) Approximately 1848 medicines are subsidised by PHARMAC, for use in the community, mostly accessible via prescription from a medical doctor.(6)

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Medicines and New Zealand

Medicines make a significant contribution to health outcomes.(7) In 2007, "Medicines New Zealand" New Zealand's medicines policy, was launched in response to access concerns from the public.(7) The aim of the policy is to promote quality, effective and optimally used medicines. To guide decisions, principles of: affordability, equity and need are stated.(7) Medicines New Zealand aims to ensure that the decisions made about prioritisation and funding are as transparent as possible, understood and open to debate. It is important for New Zealanders to have confidence that the medicine system is fair, even if they do not always agree with all of the decisions made (7).

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Pharmaceutical Management Agency (PHARMAC)

PHARMAC, established in 1993 in response to increasing expenditure on pharmaceuticals, is a separate non-profit government agency, whose role is to determine and procure, community and oncology medicines on behalf of the District Health Boards (DHBs) PHARMAC has~~ve~~ a pre-determined fixed budget which ~~they are~~ it is required to operate within. In order to provide medicines considered necessary, PHARMAC employ therapeutic and economic analyses to guide decisions. Their scope is now expanding to include hospital medicines and some medical devices. ~~Approximately $795 million and $280 million are available, for procuring community/cancer and hospital pharmaceuticals respectively.(4) This compares with a reported estimated spend of $880 million on medical devices.(5) Approximately 1848 medicines are subsidised by PHARMAC, for use in the community, mostly accessible via prescription from a medical doctor.(6)~~

For the majority of patients prescribed a medicine listed on PHARMAC's schedule, a $5 District Health Board charge is incurred. For high user or low socioeconomic patients, access enablers, such as the Community Services and High User cards and, now the Services to Improve Access (SIA) exist to help ease financial burden.(6) Additional sources of government funding include: other government agencies such as

(Accident Compensation Corporation (ACC)) local government, private medical cover and patient "out-of-pocket" co-payments. (6)

Literature exist indicating medicines issues for New Zealand related to: inequities in access, affordability, processes used and their funding. (8-31) However, no systematic work has been conducted to identify priority medicines policy issues with regards to access and funding of medicines. Within this context, it was considered timely and appropriate to conduct research that could identify priority medicines policy issues for New Zealand.

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The dataset obtained from this project was expected to be substantial and provide a solid platform contributing towards informing: medicines policy, expenditure and provision, including the development of optimal medicines management strategies.

Aim

The aim of this project was to identify priority medicines policy issues for New Zealand.

Methods

Study Design and Participant Selection

We conducted a general inductive study, using semi-structured exploratory interviews during December 2012-March 2013. Selection was purposeful, to ensure a broad representation of stakeholders and their opinions, who had one or more of the following traits in relation to medicines policy: involvement in its formation or implementation, had researched and/or commented on medicines policy, including having made submissions during its development, (n = 10); medically qualified doctor (n = 7, 4 of whom were active prescribers); medicines regulation (n = 1); representation of or, past or current involvement in medicines supply, procurement, funding or provision (excluding dispensing, n = 6); belonging to one of the ethnicities in question (n = 4); involved in medicines management (n = 9); medical information or health technology assessment interest (n = 2); medical interest group representative (past or present, n=4); private health provision and subsidy (n = 1); patient group representative (n=2). The participants characteristics are shown in table one.

A total of 26 stakeholders were contacted and explained the research involvement. Twenty stakeholders consented and interviewed. All 20 received a "Participants Information" letter, detailing the involvement, aim and general methods. All signed a confidentiality and anonymity agreement. Fifteen interviews, were conducted face-to-face and five via telephone, due to geographical or time constraints. The average length of interview ranged from 53 – 56 minutes. No gratuity was offered.

Instrument development

The main aim of this research was to identify priority medicines policy areas. An in-depth literature review was conducted, to ascertain existing information on pharmaceutical policy. A total of 105 references were identified as useful. The following broad themes were discovered and accordingly, sets of questions developed: (1) Medicines Policy: including participant's awareness, description and opinions, (2) Ethnicity inequities in accessing medicines (viz Māori, Pacifica and recently immigrated people whose first language was not English), (3) PHARMAC: its pricing policy, impact upon access, economic modelling, performance, future and any improvements (4) The Trans Pacific Partnership Agreement(TPPA): impact upon access and resultant considerations (5) High cost medicines access (6) Medicines policy issues not covered but considered important (see appendix one for question details)

The questions were piloted on one doctor of Māori ethnicity and one pharmacist with an interest in medicines policy, medicines management and academia, who has previous experience in the pharmaceutical industry. Their responses were not included for analysis.

Data Collection

Participants were encouraged to give comprehensive answers. Clarifying and confirming questions were asked where more information was considered necessary, or to avoid interviewer assumption.

All interviews were recorded on a voice recorder, transcribed intelligently (space fillers were omitted to enable ease of reading) Participants received their own transcript to proof, edit and approve. Only the approved editions were entered into INVIVO 10 (QSR International Pty Ltd) for coding.

Coding was conducted two ways: firstly, categorically according to answers and secondly, highlighted, grouped and compared – according to similarity of theme. Transcripts were checked for any missed issues.

A check for stakeholder bias was conducted using the coding summaries, no apparent bias was detected. Any variations appeared attributable to stakeholder knowledge; so were to be expected.

Results

Issues revealed specific to ethnicity, PHARMAC, the TPPA or high cost medicines are reported in those sections. A summary of issues is available in table two.

General Medicines Policy Issues

Nine participants stated they were unfamiliar with the policy. However, four demonstrated a tacit understanding. It was questioned how policy intentions and decisions are made, in the context of being achievable:

> *"How do you attain that?...what is the right way to make those overall policy decisions…" (PI)*

All participants believed medicines make a positive contribution to health. Differing levels of impact upon health were noted. There was uncertainty as to how the impact is, or could be quantified. The lost opportunity from not capturing and accessing data efficiently, was voiced by two academics for both treatment and outcomes monitoring:

> *"…we are not asking questions about patient health status before and after… so you can really see what is going on, at the GP level. Because that's at least as important as hospitalisation data". (Ac)*

Conversely, one participant said he would prefer to see more investment into epidemiology, as opposed to increasing the medicines budget, in a desire to preserve health.

Low socioeconomic patients were considered to have a higher burden of disease. Affordability to prescribers was described as the major issue, which may be compounded by the 2013 raise in prescription co-payment from $3 to $5. Australia was contrasted, where there are comparatively low prescriber and higher prescription co-payments.

Despite access enablers, such as the High User Cards and Community Services Cards, it was questioned whether those in need are utilising them. One GP said cost-sensitive patients could be managed with prudent prescribing and education on priorities:

> *"You could get all your medicines for less than a pack of cigarettes. It's educational priorities and various other things, where the effort needs to go rather than reducing the cost much further." (GP)*

The opposite situation of the misuse of access enablers was described:

> *"So it's that whole inverse law." (GP);*

> *"I initially struggled to understand how somebody could pull up outside a pharmacy in a Mercedes Benz and… present their scripts for their family and handover their Community Services Card… As soon as they get in the country; they put the money into a family trust...So the wealth of the individual gets assessed, which qualifies them for a Community Services Card and then they wave that around." (Ph)*

Sole supply[1] provision raised issues in terms of: supply outages when switching supplier (and having to pay for the alternative option), options for patient intolerance and vulnerability if a significant disruption in supply occurs (e.g., a disaster destroying a supplier's warehouse).

There was additional reference to policies and funding needing to be consistent and interlink, especially for priority areas. "Quit-Line" was given as an example: a $40 million funded smoking cessation programme, described as having markedly less evidence than the appropriate medicine (which was not funded for many years). Budgetary constraints were the reason given for this. Equally, the government funding of "alternative medicines" was described as needing debate:

> "*… government is providing funding for people to obtain alternative medicines … real debate to be had… money better spent some place else in the healthcare system?*" *(PI)*

One Doctor voiced frustration at PHARMAC's Therapeutic Advisory Committee (PTAC) [2] being *"generalists"* who over-ride the recommendation of their subcommittee. With patient sub-typing and genomic medicine on the horizon, he considered *"generalists"* may not understand what they are assessing and dismiss research, thereby inhibiting access. Access is then through an ability to pay for litigation and decided by a non-medical expert.

With demographic changes increasing demand for healthcare services and a general movement towards increasing costs of new medicines, there was concern for future affordability. Suggestion was funding may move away from being population based, toward funding health outcomes. The biggest concern being: the discovery and affordability of a panacea. An academic suggested: changes in co-payments, taxation or medicines classification status may result.

The oncologist had concerns that the lack of research (research being an attracter) being conducted within New Zealand, will compound the low availability of future prescribers. Extending prescribing ability to non-doctors was considered to help. However, for oncology, a medical specialist was considered to still be required to make treatment decisions.

Ethnicity Issues

Most issues presented, related to socio-economic variables and are presented under General Medicines Policy Issues.

Those with poor English speaking skills were described as having access to an English speaking relative or even interpreters if needed:

> *"I think if they can access General Practice or the Hospital system, their access to the medications is just as good as anybody else's. I'm not aware of any specific ethnic problems in accessing our medicines.." (GP)*

[1] Sole supply arrangements are likely to be used by PHARMAC in markets where generic competition exists, resulting in there being only one brand of a particular chemical listed. It is possible that PHARMAC would agree preferred supplier status for some chemicals in exchange for price concessions, affecting access to related pharmaceuticals within the same therapeutic group.(32. Pharmaceutical Management Agency. Proposed pricing strategy initiatives - sole supply arrangements. Pharmaceutical Management Agency; 2002 [cited]. Available from: http://www.pharmac.govt.nz/2002/07/19/nhps.pdf.

[2] PTAC is PHARMAC's primary clinical advisory committee. PTAC's role is to provide clinical advice to the Board of PHARMAC.

One GP felt strongly that Māori and Pacifica access inequities are evidenced by poorer health outcomes. He considers his colleagues are treating everyone the same but with inequitable risk: earlier intervention~~; and~~, improved communication, education and patient engagement are required.

> *"I think the key issue is the prescribers have a poor understanding of inequalities. Because, the prescribers generally approach things as; I treat everyone the same… they must have an inequity lens on anyone they see… but if the quality of your discussion and the quality in the way in which you prescribed that was poor i.e., you culturally are incompetent and you have a disconnect with the patient…" (GP)*

Other issues related to Asian ethnicities wanting treatment (oncology setting) irrespective of likely outcomes and, the use of alternative treatments e.g., St John's Wort or Vitamin C injections impacting upon medical treatment. One of the doctors had issue with alternative practitioners recommending such treatments as safe and evidence based, upon requesting information to support these treatments, he found the paper to be an out-dated and flawed case study.

Pharmaceutical Management Agency (PHARMAC)

There was general appreciation shown towards PHARMAC's strategy of creating competition in order to achieve a lower purchasing price. This was seen as advantageous for the purchasing of a greater range of medicines, in the context of a fixed budget.

The budget was defined as the threshold for provision, which was considered too small by an academic and PI, causing a focus on cost as the driver of value and provision, thereby contributing toward *"static efficiency"*:

> *"If Pharmac's objective is to stay within budget then it's doing well… improve the health of New Zealanders within a capped pharmaceuticals budget…it's doing moderately well…objective were to improve the health of New Zealanders taking into account the financial constraints of Vote Health…it's doing poorly because it should be fighting for a better share of Vote Health." (Ac)*

A public servant made the following comment: *"You can always achieve more with more."* In terms of a bigger budget but there isn't an analytical framework in place which would define whether the medicines budget receives a fair proportion of "Vote Health."

There was concern: whilst PHARMAC's budget is determined at regular defined intervals, medicines enter the marketplace sporadically, for which funds may not be available.

The private health care provider (PHCP) thought PHARMAC's approach of requiring new and more expensive medicines to be better than standard medicines a: *"completely acceptable approach."* It was suggested by the PHCP and an academic that their approach could be more widely adopted, both overseas and with the expansion of PHARMAC's role to medical devices:

"…the expertise PHARMAC has built up…is something that we could learn from and borrow from, for the wider health sector… I'd like to see them take on medical devices, because that is absolutely scandalous that these products are getting onto the market without being properly evaluated…" (Ac)

There was caution given from one pharmacist that PHARMAC's expansion into hospital medicines (in an acute care setting of moribund disease) may limit choices. Concern was shown for risk, if New Zealand is world-leading in this type of provision.

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Provision was described as having a utilitarian focus: *"The greatest good for the greatest number" (PS)* and described as being: you get what you need - not what you want. One doctor questioned whether the lost opportunity from not treating someone is being measured. Rare disorder patients were mentioned and are discussed under High Cost Medicines. The distinction was made that provision of a medicine in a cost effective manner, which PHARMAC achieves, is not the same as delivering healthcare:

> *"I find some of their PR a little bit irritating…bray on about the marvellous healthcare they're delivering… delivering medicines in a cost effective manner but that's not saying it's delivering healthcare…" (PHCP)*

The question of the economic modelling Pharmac undertakes received very favourable comments from 15 of the participants. Three participants were not familiar with economic modelling:

> *"I think it's world leading actually. No one else dares do it. That's the crazy thing. Here we are little old New Zealand and we dare do it." (Ac);*
>
> *"Well I mean, as a tax payer you could argue that for the majority of the products they get in, they've done a really good job of driving cost out of the system." (PI);*
>
> *"Technically it's very good. PHARMAC considers clinical effectiveness and cost effectiveness.. they make trade-offs… they look at the QALYs and the number of people affected and how their quality of life will be improved and so on, I think is a very good model." (PS)*

It was suggested that cost-benefit should be a consideration because of valuing the return of an individual back to their normal daily activities, such as what Accident Compensation Corporation does in assessing intervention options.

A pharmaceutical industry representative and academic were concerned the required economic modelling submitted by suppliers is adjusted with unknown *"in-house"* variables, making it hard for suppliers to understand decisions. This was contrasted against Medsafe's practice, where decision modelling is transparent:

> *"Pharmac receives a dossier from the company…Assumptions of statistical models get changed…QALYs get changed…population who will use the product get changed…that should be part of a scientific debate…companies don't know what information is being used to make the decisions on their products…we would like a right of reply to those… It happens with MedSafe…Not as though it could potentially negatively affect evidence based decisions."(PI)*

Delays in the submission process of up to eight years and described as a: *"medicines waiting list,"* were of concern for an academic, pharmaceutical industry representative and patient group representative, who all thought access was related to cost. There was suggestion from one academic to follow Australia's submission process and out-source assessments from independent bodies.

Trans Pacific Partnership Agreement (TPPA)

Very few participants were familiar with the TPPA, some referred to speculation and no facts. One participant refused to give any comments related to the TPPA.

There was acknowledgement that trade deals are complex and often require compromises and trade-offs. New Zealand was referred to as a: *"small country"* and *"we need our trade partners."* There was concern that already "*big amounts"* are being spent on healthcare and the *"benefits are low"* and, if there is a resultant increase in the cost of medicines, where would resources come from, to off-set any cost increases.

The main issues were: (1) Patent extension, delaying generic entry to market, thereby prolonging a higher cost of provision; (2) Industry influencing supply (described as an issue of sovereignty) may result in quicker access to new medicines but also an increase in public campaigns and appeals processes if PHARMAC's decisions are unpopular with the industry or patient groups; and (3) The call for transparency in PHARMAC's assessment process caused the most concern and confusion. One Academic said he didn't think PHARMAC could be more transparent and that transparency might mean the industry discloses its pricing processes and the results of all clinical trials.

In general, scepticism was voiced as to what the driving force is behind the agreement and what the benefits would be for New Zealand - with the USA being a protected market (heavily subsidised) Australia was described by a pharmaceutical industry representative, as getting *"trounced"* over their agreement with the USA, losing a lot of their pharmaceutical production and jobs as a result:

> *"Forget it…wouldn't even bother going along to the negotiations"(PHCP)*;
>
> *"…tell the US to bugger off quite frankly. You either put everything on the table and we talk about it or no, you don't…We should learn from what happened in Australia…" (PI)*

Conversely, another participant suggested whilst *"America"* has influence, it may become limited as a result of the influence of China's developing economy and differing ideas around protection and, new opportunities may develop:

> *"…a hugely developing economy in the form of China that basically has total disregard for such things… so the ability for America…is probably going to be limited in the world of the future, and maybe different forms of protection of ideas will kind of evolve… it's very hard to predict how the market might respond or what kind of new opportunities develop." (PS)*

One academic suggested that PHARMAC's monopsony is an anathema to the USA. A pharmaceutical industry representative said Medicines New Zealand (New Zealand's prescription medicines representative association, same title as the policy) is attempting to ensure its USA equivalent understands New Zealand's medicines system:

> "*…working quite hard to ensure…our sister organisation in the US is effectively asking the US government to achieve out of the process, is well enough informed to understand actually what the New Zealand model does achieve, what it doesn't achieve and how that can be improved…So we're working hard to make sure it's a process that actually benefits New Zealanders as well, and all of the transparency, timeliness, appeals – those aspects that we've discussed, are exactly I think what the US is likely to be asking for.*

Most considered that New Zealand's current ability to access generic medicines or, independence in procuring medicines should be upheld. If not, funds may need to be redirected from other services or, patient co-payments would need to rise, in order to compensate a likely increase in the cost of medicines.

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High Cost Medicines

A GP questioned the necessity of continuing the Special Authority (SA)[3] status for a medicine, once the appropriate use of a medicine has been established. Not all participants were familiar with the Named Patient Pharmaceutical Assessment (NPPA)[4] access scheme. Most high cost medicines were described as being *"breakthrough"* or *"expensive"* and are restricted, to control spending. One participant said, if it was *"dirt cheap,"* there would be "no argument," indicating the case even if the medicine didn't have clear health benefits:

> *"My bet, is that PHARMAC would listen to anyone that agrees with them saying no. Because it's expensive ....They are diametrically opposed for a reason and the reason is cost." (Ph);*

> *"The Rabbits in charge of the lettuce patch" (M)*

One public servant thought there to be no inappropriate blocking of access to medicines, as no complaints about access have been received at their level. Equally, another Public Servant commented that there are patients accessing medication costing up to $500, 000 per year:

> *"…So it's not that the system can't cope with treatments that are high cost, it's just that we would expect a return for that cost and for it to be justifiable in terms of what we value." (PS)*

A small group of patients were described as not having access to high cost medicines. Access was described as *"the collective good."* Conversely, *"people dying from a lack of access to very cheap and simple therapies"* were described. It was suggested that it is a DHB's remit to look after its population, highlighting the issue of population versus individual access. A statement was made: are we advocating treatment at any cost and if so, who pays:

> *"It's a question of who pays for all these things. I think if you have pretence; like there is in the USA, that cost isn't of any relevance… then you're going down the wrong path." (PHCP)*

The oncologist described the NPPA process as inefficient: a comprehensive and referenced application, takes him up to 6 hours, potentially impacting on his clinic time and perversely hindering patient access. He suggested PHARMAC at a nominal cost could employ someone to aid in information gathering and in the process develop expertise.

Additionally, the oncologist believes oncology has the stigma that everyone dies but individual survival may be greater than the median survival assessment. This issue was presented in comparing the availability of 2-3 drugs in Australia - unavailable in New Zealand.

[3] Special Authority criteria define the clinical circumstances of patients who can receive funding for the medicine. People may first be required to try a less expensive medicine or the medicine may need to be prescribed by a particular type of health practitioner.

[4] NPPA is a mechanism to give individual named patients access to medicines they need, but which aren't funded on the Pharmaceutical Schedule. NPPA replaces the three Exceptional Circumstances (EC) schemes that PHARMAC previously managed.

Questions were posed: (1) Is it fair to give 4th or 5th line chemotherapy and not give a first line treatment e.g. for rare disorders? and, (2) When do you stop treatment, a patient was described as gaining access to expensive medication, their condition was fragile and they died a few weeks later:

> *"I think it's important if Pharmac has a few loose strings in terms of hospital and severe rare conditions. They are perhaps because of how they are funded, they want a very narrow perspective on those, to try and avoid blow out. They are very emotive issues we don't always know how to best manage people's care. "* (Ph)

High need patients, such as rare disorder patients received the most sympathy for difficulty in access because of the exclusion criteria. Evidence requirements were described as difficult to attain due to low patient numbers. Conversely, the PHCP suggested the supplier needs to produce quality evidence:

> *"…where there's some evidence, but not solid or quality evidence: … the company doing the – providing the medication, it behoves on them to do some research in those areas and produce quality data."(PHCP)*

Discussion

We purposefully attempted to be open to issues and their capture, despite some issues already being identified. Our focus was on access to medicines. It is possible there are other issues in existence, we neither recognised, nor captured. We did not seek to determine issues specifically related to generic medicines, considered a "*vital component of New Zealand's medicine cost management policies*" by Babar et al.(12) ~~Apart from the sole supply issues (which encompasses generic medicines) we additionally found a lack of palatability, from a brand of paracetamol not being coated.~~

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Medicines Policy

~~The significance of the lack of familiarity shown with "Medicines New Zealand" is uncertain but better familiarity with policy and processes of evaluation may be required if the goals are to be fulfilled.~~

Medicines are clearly valued health interventions: evidenced by~~from~~ the budget, literature and responses from stakeholders. The smaller percentage spend on pharmaceuticals in New Zealand (described as a constrained budget) compared with similar countries such as Australia, UK and USA,(2) may in fact reflect the price reduction strategies that are implemented by PHARMAC(32), as opposed to less opportunity to improve health outcomes. However, this needs to be tested through robust research on health outcomes and their relationship to pharmaceutical spending.

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Delayed access and the resultant impact discussed by some of the participants, was also described by Ellis and Hamer,(33) in relation to New Zealand's statin availability for atherosclerotic patients, as probably negatively impacting health outcome and considered to be due to the capped budget. They considered this *"anomalous,"* as other types of health care are not capped. This anomaly was also described by a number of participants but may change with PHARMAC's expanding role.

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New medicines are increasing in costs along with demand, causing tension in affordability. Price efficiency initiatives, such as what PHARMAC encourages, help ease the tension in affordability of provision. Another option is to reduce demand: either through gate keeping (not usually a popular choice) or genuine effects, such as initiatives to maintain health or prevent disease. We assume a reduction in demand and therefore

burden of provision, should result in healthcare becoming more affordable, for providers and helping those remaining in need.

~~Resolving disputes between the experts and provision were described as being dependent upon an individual's ability to pay for litigation - not an equitable process and one where a non-medical expert makes the decision~~. Manning (34) compared the processes of: decisions, pricing, economic analysis, provision and access and, participation and appeals between the UK, Australia and New Zealand. It was suggested that resolving issues may benefit from a disputes panel comprising a broad range of experts in: scientific, economic, policy and ethical evaluations, in order to provide an objective decision. Manning additionally reported that approximately one sixth of the United Kingdom's National Institute for Healthcare and Clinical Excellence (NICE) recommendations are appealed and upheld. (35) There shouldn't be great demand, if evaluation processes are robust.

Ethnicity

Our participants revealed that low socioeconomically related populations (encompassing Māori and Pacifica people) are continuing to have access issues related to financial, structural, educational and cultural barriers. These findings were consistent with that of Jatrana et al (36) who assessed SOFIE-health's 18320 respondents (an add-on to Statistics New Zealand's longitudinal survey of Family, Income and Employment) Māori and Pacific people were more likely to defer purchasing a prescription due to cost, which at that time was $15.

Māori represent approximately 15 percent of New Zealand's population(37) and on average have the poorest health status of any ethnic group in New Zealand.(38, 39) Pacifica people represent 6.5 percent of the population and also experience health inequalities. He Korowai Oranga: the Māori Health Strategy (2002),(38) recognises the Treaty of Waitangi principles of: partnership, participation and protection, through which, the aim is to reduce existing health inequalities. This aim is extended to include the Pacific people, like Māori, they are over represented by a low socioeconomic situation, reflecting low affordability and health literacy, which in turn affects access. The Ministry of Health recently launched Services to Improve Access(SIA),(40) an additional targeted capitation payment, available to Primary Healthcare Organisations(PHOs) to reduce health inequalities. It is designed for new services (e.g., outreach programmes) or improving access (e.g., funding transport) for Māori, Pacific people and those of low socioeconomic status. Once SIA is embedded, it would be prudent to evaluate its impact.

Other Ministry of Health initiatives, such as Whanau Ora (to build the health, participation and capability of families) and, One Heart Many Lives (to improve the cardiac health of Māori and Pacific men) along with recent changes in health practitioner training, appear good initiatives for engaging Māori and Pacifica in a culturally appropriate way. It would be prudent to evaluate their impact.

We did not have any issues specifically described for new immigrants. It was described to us that patients with poor English speaking capability, present to practitioners with an English speaking person, or frequent a surgery of their ethnicity. This is at odds with Babar et al,(9) who found for 11 Chinese and Indian migrants, residing in New Zealand for less than five years, financial barriers existed in affording doctors, pharmacists and medicines and, that language barriers exist. This anomaly may highlight the differences in both the perspective and experience of the stakeholders we interviewed.

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Asia and India have different medicines access systems to New Zealand. Babar additionally found there is a lack of information on New Zealand's medicines system, provision and classifications. The United Nations and World Health Organisations, when discussing the right to health,(41) refer to migrants as being vulnerable to reduced access to health services for reasons that include: language or cultural barriers. New Zealand has a significant migrant population, reported as 927 000 in 2006,(42) The current main countries for immigration are: China (15%), United Kingdom (unspecified), India (13%) and the Philippines (8%).(42) In consideration of Babar et al's work and immigration statistics, there may be a significant number of people from these countries with issues, resulting in difficulties in accessing healthcare and therefore medicines.(8-10) In light of this, it may be worth further investigating new immigrant issues.PHARMAC

The general appreciation for New Zealand's need to be efficient to provide more medicines expressed by our stakeholders, was also shown by Ragupathy et al.(26) Included, was the need to apply consistent economic evaluations to other health technologies, to support congruous decisions for resource allocation. PHARMAC's expansion into procuring hospital medicines and medical devices may enable greater consistency of evaluation across technologies.

The significance of PHARMAC's role expansion should not be underestimated. PHARMAC will need to practice caution with expanding their role into hospitals, which are generally settings of acute and moribund disease. We are unaware whether a closed formulary has occurred elsewhere in the world. PHARMAC have been noted to have consulted directly with medical specialist groups to discuss their role expansion, including consultation on hospital medical devices,(43) so would appear to be fully cognisant and appreciative of this issue.

We found delays of up to eight years, in PHARMAC's process for funding medicines onto the Pharmaceutical Schedule, which our stakeholders purported to be due to the medicine's pricing and/or PHARMAC's budget not being able to expand. Other reasons may be the medicine's priority status, insufficient information or, not meeting PHARMAC's nine decision criteria.(44) The question is whether this means delays in therapeutic advancement and therefore improved health outcomes.

The measurement of opportunity foregone was of clear concern to the Oncologist we interviewed. New Zealand has a capped medicines budget, it cannot expand and therefore drives the need for efficient spending (determined using cost utility analysis, where medicines are assessed against QALY gains per $1 million) Using this process for provision means there is opportunity foregone, as described by Milne and Wonder.(14) We are not aware of New Zealand focused research assessing either opportunities foregone or, other specialist viewpoints on access. The exceptions being: Ellis and Hamer(33) in 2008, discussing the delayed availability of cardiac medicines; MacCormack et al in 2009,(31) assessing stakeholders views on needed access to high cost medicines and; The Sage report for the Ministry of Health in 2010, (22) reporting the consultation of stakeholders on the proposal to expand PHARMAC's role.

Sole supply issues (supply outages, lack of palatable formulations, resultant out of pocket payments for alternatives and vulnerability as a result of a disaster) were reported as still continuing, despite there being penalties for suppliers. This was also reported by Babar et al,(45) who reported additional concerns with poor quality products in the past, from previous studies.

Trans-Pacific Partnership Agreement (TPPA)

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There is very little information available on the TPPA, the reason given: particulars of the negotiation are changing. What does exist concurs with our findings: it questions the motivation and self-interest of parties involved and warns of possibly binding impacts that may affect health services budgets and PHARMAC's autonomy, including method of procurement and provision.(25-29, 46) Such impacts stem from the USA's desire for stricter protection of intellectual property rights, transparency of in-house evaluation, regulatory coherence, dispute settlement, government procurement and evidence based decisions being contestable in court. Unless budgets are expanded to cope with likely increases in costs, there may need to be a re-evaluation of provision, subsidies and co-payments. In contrast to existing publications, our research additionally suggested a TPPA may enable earlier access to newer medicines. It may be of use to quantify what effect a TPPA would have upon medicines access.

High Cost Medicines

A significant issue discussed in our study, was the need to differentiate between high cost medicines and highly specialised needs or medicines in relation to the NPPA access scheme. McCormack et al,(31) suggest a medicine that costs $20 000 per patient per year may be considered high cost. It is important to be cognisant of the total cost to the health system of any medicine, which is dependent upon the number of patients treated (volume used) and the acquisition price. Some high cost medicines may not result in a high total cost to PHARMAC, for some patient groups. Gallego et al,(47) question how treating large populations at high total cost for small population gains, compares with treating smaller populations, for possibly significant benefit.

The issue of treating large versus small populations may intensify with patient subtyping and genomic medicines development (as described by the oncologist) where greater expectation to fund (i.e., demand) may occur. With the NPPA process now reported in our findings, as enabling the capturing of cancer patient subtype information, cancer medicines outcomes may become easier to measure and if positive, make it harder to decline funding treatments. It may also mean the table is turned and large populations end up having limited treatment options, if outcomes cannot be measured in the same way. However, funding outcomes will give a clear indication for innovation and direction to both suppliers and funders of medicines.

Our findings describe both the SA and the NPPA access schemes as being inefficient. The SA inefficiency finding is also supported by Babar et al's evaluation of GP perceptions on access to medicines in New Zealand.(45) Once correct prescribing of a medicine has been established it may not be necessary to continue a medicine's SA status. The NPPA process appears to impact significantly on consultant clinic time which may perversely hinder patient access. With demographic trends indicating greater demand for such medicines, the impact of the inefficiency may intensify. PHARMAC's website lists 555 approvals and 15 declines for NPPA access.(48) It may be more efficient for PHARMAC, at a nominal cost, to contract an evidence based facilitator, to ease the burden of application for clinicians.

Difficulty in access to high cost medicines, encompassing rare disorder patients, as described by our participants, has been widely documented.(31, 47, 49-58) The nature of rare diseases makes it hard to gain the necessary evidence PHARMAC requires for evaluation. This issue is compounded for suppliers because the need to satisfy both manufacturer ordering and regulatory requirements, adds to the unit cost of supply for low volume demand medicines. It may be worth investigating options to reduce cost of supply and provision in the context of constrained evidence. PHARMAC have recently sought public and professional input into its decision criteria. The results have yet to be published but may reveal new options or initiatives.

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Our research highlighted the issue of access to medicines of therapeutic value in the context of a fixed predetermined budget and the difficulties in how priorities for funding are determined. Lu et al (59) in discussing ethical perspectives to the access of high cost medicines in Australia, discussed the issue of having equal need requires equal opportunity to access care and suggest where evidence requirements are not achieved, treatment commence on a trial and outcome basis. This does come with ethical concerns but may enable both access and capturing evidence. MacCormack et al (31) suggest "risk sharing" supply to ensure some form of access (defining a threshold for maximum numbers to treat for a high cost medicine, above which, the supplier funds)

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Conversely, Simoens et al (52) caution providing access to medicines with limited effectiveness, implies rare disorders health improvement is more valuable than a common disease, which challenges the utilitarian view of: the health gain of each patient is valued equally. With both increasing effort in the development and availability of orphan drugs, this issue may only worsen. Equally, other questions arise: because we see the ill health, can't mean preferential treatment over someone who has a "silent" state of declining health. There are people not getting access to inexpensive medicines, who are at risk, as stated by an academic. Perhaps remedying issues of access based on need, could start with prioritising based on the impact of an unmet need?

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Conclusion

Overall, despite issues being identified, there was reasonable satisfaction with the New Zealand Medicines Policy and its principles. In particular that provision is evidence based, cost effective and there is equitable ability to have prescribed medicines listed as funded, on PHARMAC's schedule.

However, despite this, there appears to be some patient groups still experiencing difficulties in access, not necessarily appearing as a result of medicines policy or PHARMAC. Such groups being: rare disorders and the low socio economic (encompassing rural, Māori and Pacifica populations) Other issues ranged from: the pharmaceutical industry's pricing of new medicines, manufacturer and registration requirements, the submission for funding process, increasing demand and costs, budgetary constraints, cultural and health literacy, patient affordability and access to prescribers, -through to knowledge development for clinical expertise and the measurement of health outcomes.

Our study has highlighted issues in access based upon need and the consequences of unmet need. The context being: a fixed, predetermined budget and increasing demand, is causing constraints in affordability. We suggest these issues and consequences of unmet needs may worsen and options for demand and provision may need to be explored further.

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Ethics: Ethics approval was obtained from The University of Auckland, Ethics Committee. Approval number; 8367

Authors contribution: ZB conceptualised and designed the study. The data collection, entry and analysis was handled by SF and ZB. SF and ZB wrote the manuscript. The final version is approved by all authors. ZB acts as an overall guarantor to this study.

Transparency: ZB affirms that the manuscript is an honest, accurate, and transparent account of the study being reported; that no important aspects of the study have been omitted.

Provenance and peer review: Not commissioned; externally peer reviewed.

Data sharing statement: The original data are available from the principal author (ZB).

Table one: Stakeholder Characteristics:

Stakeholder Group	Number (n)	Professional Title/Characteristics~~Area of Professional Group/Title~~ (n)
Academia (Ac)	3	Sociologist (2) Pharmacoeconomist (1)
Public Service (PS)	5	Politician (1) Medsafe (1) Policy Analyst (1) DHB Planning (1) Pharmac (1)
Medicine (M)	4	Oncologist (1) General Practice (3)
Pharmacist (Ph)	3	DHB (2) Community based (1)
Pharmaceutical Industry (PI)	2	Manufacturing (1) Representative (1)
Patient Group Representative (PGR)	2	Long Term Conditions (2)
Private Health Care Organisation (PHCO)	1	Medical doctor (1)

References

1. Statistics New Zealand. Top Statistics. 2013 [cited]. Available from: http://www.stats.govt.nz/browse_for_stats/snapshots-of-nz/top-statistics.aspx.
2. Organisation for Economic Co-operation and Development countries. OECD Health Data 2013 - frequently requested data: health expenditure. 2013 [cited]. Available from: http://www.oecd.org/els/health-systems/oecdhealthdata2013-frequentlyrequesteddata.htm.
3. Ministry of Health. Funding. Ministry of Health; 2013 [cited 2013]. Available from: http://www.health.govt.nz/new-zealand-health-system/funding.
4. Pharmaceutical Management Agency. Setting and managaing the combined pharmaceutical budget (CPB). New Zealand Government; 2013 [updated 2013; cited 2013]. Available from: http://www.pharmac.health.nz/ckeditor_assets/attachments/381/infosheet-08-community-pharm-budget-2013.pdf.
5. Pharmaceutical Management Agency. PHARMAC and hospital medical devices. 2013 [cited]. Available from: http://www.pharmac.health.nz/medicines/hospital-devices/.
6. Pharmaceutical Management Agency. New Zealand Pharmaceutical Schedule. 2013 [updated April, 2013; cited 2013]. Available from: http://www.pharmac.govt.nz/2013/04/03/Sched.pdf.
7. Associate Minister of Health, Minister of Health. Medicines New Zealand: Contributing to good health outcome for all New Zealanders. In: Health Mo, editor. Wellington, New Zealand: Ministry of Health, Wellington, New Zealand; 2007.
8. DeSouza R, Garret N. Access Issues for Chinese People in New Zealand Final Report. : Auckland University of Technology, Auckland; 2005 2005. Contract.
9. Babar ZUD, Pengelly K, Scahill SL, Garg S, Shaw J. Migrant health in New Zealand: Exploring issues concerning medicines access and use. Journal of Pharmaceutical Health Services Research. 2013;4(1):41-9.
10. Suin T. An holistic approach to Asian health Fourth internaitonal Asian health and well-being conference; 2010; Auckland. 2010.
11. Ragupathy R, Aaltonen K, Tordoff J, Norris P, Reith D. A 3-dimensional view of access to licensed and subsidized medicines under single-payer systems in the US, the UK, Australia and New Zealand. PharmacoEconomics. 2012;30(11):1051-65.
12. Babar ZUD, Grover P, Stewart J, Hogg M, Short L, Seo HG, et al. Evaluating pharmacists' views, knowledge, and perception regarding generic medicines in New Zealand. Research in Social and Administrative Pharmacy. 2011;7(3):294-305.
13. Wonder M, Milne R. Access to new medicines in New Zealand compared to Australia. New Zealand Medical Journal. 2011;124(1346):12-28.
14. Milne R, Wonder M. Response to PHARMAC on access to new medicines in New Zealand compared to Australia. New Zealand Medical Journal. 2011;124(1347):18.
15. Tordoff JM, Norris PT, Reith DM. "Price management" and its impact on hospital pharmaceutical expenditure and the availability of medicines in New Zealand hospitals. Value in Health. 2008;11(7):1214-26.
16. Aaltonen K, Ragupathy R, Tordoff J, Reith D, Norris P. The impact of pharmaceutical cost containment policies on the range of medicines available and subsidized in Finland and New Zealand. Value in Health. 2010;13(1):148-56.
17. Cumming J, Mays N, Daubé J. How New Zealand has contained expenditure on drugs. BMJ (Online). 2010;340(7758):1224-7.
18. Metcalfe S, Grocott R. Comments on "simoens, s. health economic assessment: A methodological primer. int. j. environ. res. public health 2009, 6, 2950-2966"-New Zealand in fact has no cost-effectiveness threshold. International Journal of Environmental Research and Public Health. 2010;7(4):1831-4.
19. Moodie P, Metcalfe S, Poynton M. Do pharmaceutical score cards give us the answers we seek? New Zealand Medical Journal. 2011;124(1346):69-74.
20. Moodie P, Metcalfe S, McNee W. Response from PHARMAC: Difficult choices. New Zealand Medical Journal. 2003;116(1170).

Field Code Changed

21. Grocott R. Applying Programme Budgeting Marginal Analysis in the health sector: 12 years of experience. Expert Review of Pharmacoeconomics and Outcomes Research. 2009;9(2):181-7.
22. Sage D. Report on the consultation period for the proposal to expand the functions of PHARMAC; 2010. Contract.
23. Morgan S, Boothe K. Prescription drug subsidies in Australia and New Zealand. Australian Prescriber. 2010;33(1):2-4.
24. Carlson JJ, Sullivan SD, Garrison LP, Neumann PJ, Veenstra DL. Linking payment to health outcomes: A taxonomy and examination of performance-based reimbursement schemes between healthcare payers and manufacturers. Health Policy. 2010;96(3):179-90.
25. Gleeson D, Lopert R, Reid P. How the Trans Pacific Partnership Agreement could undermine PHARMAC and threaten access to affordable medicines and health equity in New Zealand. Health Policy. 2013.
26. Ragupathy R, Tordoff J, Norris P, Reith D. Key informants' perceptions of how pharmac operates in New Zealand. International Journal of Technology Assessment in Health Care. 2012;28(4):367-73.
27. Moynihan R. New Zealand agency comes under pressure to pay more for drugs. BMJ (Clinical research ed). 2011;342.
28. Foster RH, Wilson N. Trans-pacific partnership: Will Pharmac become a victim of its own success? BMJ (Online). 2011;343(7818).
29. Faunce TA, Townsend R. The Trans-Pacific Partnership Agreement: challenges for Australian health and medicine policies. Med J Aust. 2011;194(2):83-6.
30. Raftery JP. Paying for costly pharmaceuticals: regulation of new drugs in Australia, England and New Zealand. Med J Aust. 2008;188(1):26-8.
31. MacCormack P, Quigley J, Hansen P. Review of access to high-cost, highly specialised medicines in New Zealand. ; 2010. Contract.
32. Pharmaceutical Management Agency. Proposed pricing strategy initiatives - sole supply arrangements. Pharmaceutical Management Agency; 2002 [cited]. Available from: http://www.pharmac.govt.nz/2002/07/19/nhps.pdf.
33. Ellis CJ, Hamer AW. Cardiovascular health in New Zealand: areas of concern and targets for improvement in 2008 and beyond. New Zealand Medical Journal. 2008;121(1269):5-10.
34. Manning J. Priority-setting processes for medicines: the United Kingdom, Australia and New Zealand. J Law Med. 2011;18(3):439-52.
35. National Institute for Health and Care Excellence, . [cited]. Available from: http://www.nice.org.uk.
36. Jatrana S, Crampton P, Norris P. Ethnic differences in access to prescription medication because of cost in New Zealand. Journal of Epidemiology and Community Health. 2011;65(5):454-60.
37. Statistics New Zealand. Māori population estimates. 2012 [cited]. Available from: http://www.stats.govt.nz/browse_for_stats/population/estimates_and_projections/maori-population-estimates.aspx.
38. Ministry of Health NZG. He Korowai Oranga: Māori Health Strategy, . 2002 [cited]. Available from: http://www.health.govt.nz/publication/he-korowai-oranga-maori-health-strategy.
39. Ministry of Health NZG. The Health of Māori Adults and Children. 2013 [cited]. Available from: http://www.health.govt.nz/publication/health-maori-adults-and-children.
40. Ministry of Health NZG. Services to improve access. 2011 [cited]. Available from: http://www.health.govt.nz/our-work/primary-health-care/primary-health-care-services-and-projects/services-improve-access.
41. The United Nations WHO. The right to health. 2008 [cited]. Available from: http://www.who.int/hhr/activities/Right_to_Health_factsheet31.pdf.
42. Ministry of Business Innovation and Employment. New Zealand Government. Migration research. [cited]. Available from: http://dol.govt.nz/research/migration/monthly-migration-trends/13jun/index.asp.
43. Pharmaceutical Management Agency. Adapting the PHARMAC model for medical devices management,. 2013.
44. Pharmaceutical Management Agency. Decision criteria Pharmac; 2013 [cited 2013 11.4.13]. Available from: http://www.pharmac.health.nz/medicines/how-medicines-are-funded/decision-criteria.
45. Babar ZUD, Grover P, Butler R, Bye L, Sheridan J. A qualitative evaluation of general practitioners' perceptions regarding access to medicines in New Zealand. BMJ Open. 2012;2(2).
46. Kelsey J. Hidden agendas what we need to know about the TPPA. BWB texts; 2013 [cited. Available from: www.bwb.co.nz.

47. Gallego G, Taylor SJ, Brien JaE. Funding and access to high cost medicines in public hospitals in Australia: Decision-makers' perspectives. Health Policy. 2009;92(1):27-34.
48. Pharmaceutical Management Agency. NPPA outcome report cumulative for March - December (updated 24/1/13). Pharmaceutical Management Agency,; 2013 [cited]. Available from: http://www.pharmac.health.nz/ckeditor_assets/attachments/249/summary_of_nppa_decisions_dec_12.pdf.
49. Forman J, Taruscio D, Llera VA, Barrera LA, Coté TR, Edfjäll C, et al. The need for worldwide policy and action plans for rare diseases. Acta Paediatrica, International Journal of Paediatrics. 2012;101(8):805-7.
50. Lu CY, Williams K, Day R, March L, Sansom L, Bertouch J. Access to high cost drugs in Australia. British Medical Journal. 2004;329(7463):415-6.
51. Lee TH, Emanuel EJ. Tier 4 drugs and the fraying of the social compact. New England Journal of Medicine. 2008;359(4):333-5.
52. Simoens S, Cassiman D, Dooms M, Picavet E. Orphan drugs for rare diseases: Is it time to revisit their special market access status? Drugs. 2012;72(11):1437-43.
53. Munoz J, Kurzrock R. Targeted therapy in rare cancers-adopting the orphans. Nature Reviews Clinical Oncology. 2012;9(11):631-42.
54. Hudson I, Breckenridge A. The challenges of orphan drugs and orphan diseases: Real and imagined. Clinical Pharmacology and Therapeutics. 2012;92(2):151-3.
55. Drummond MF, Wilson DA, Kanavos P, Ubel P, Rovira J. Assessing the economic challenges posed by orphan drugs. International Journal of Technology Assessment in Health Care. 2007;23(1):36-42.
56. Joppi R, Bertele V, Garattini S. Orphan drugs, orphan diseases. The first decade of orphan drug legislation in the EU. European Journal of Clinical Pharmacology. 2013;69(4):1009-24.
57. Largent EA, Pearson SD. Which orphans will find a home? The rule of rescue in resource allocation for rare diseases. Hastings Center Report. 2012;42(1):27-34.
58. Cheema PK, Gavura S, Migus M, Godman B, Yeung L, Trudeau ME. International variability in the reimbursement of cancer drugs by publically funded drug programs. Current Oncology. 2012;19(3):e165-e76.
59. Lu CY, Macneill P, Williams K, Day R. Access to high cost medicines in Australia: Ethical perspectives. Australia and New Zealand Health Policy. 2008;5.

Appendix One

Questionnaire

1. Medicines Policies
   - What contribution medicines make to the health status of New Zealand(ers) ?
   - Awareness of "Medicines New Zealand"; the New Zealand Medicines Strategy ?
   - The impact of Medicines Policy upon access to medicines ?
   - What if anything, could be done to improve Medicines Policy; Why and How?

2. Medicines Access and Inequalities/Inequities on the basis of Ethnicity
   - What is their view of medicines access and inequalities based on; the identified ethnicities ?
   - What if anything, could be done to improve access and inequalities for these ethnicities ?

Note: the ethnicities were described as: Māori, Pacifica, Indian or Asian, or such people where English may not be a first language

3. Pharmac
   - Awareness of Pharmac's pricing policy ?
   - Description of Pharmac's pricing policy ?
   - Awareness of how Pharmac subsidises and funds medicines ?
   - Pharmac's impact upon access ?
   - Opinion of Pharmac's model of pricing in terms of cost effectiveness, cost utility and reference pricing ?
   - How well Pharmac is performing it's role, what impact has it had ?
   - What is the future for Pharmac, in next 5,10,20 years. What could be the likely issues ?
   - What if anything, could be improved in relation to Pharmac ?
   -

4. Transpacific Partnership Agreement (TPP)
   - With the likely TPP agreement with United States of America; what impact will it have on medicines procurement and availability and, why ?
   - What needs to be considered with the TPP and access to medicines ?

5. Accessing and Funding of High Cost Medicines
   - Awareness of the accessing and funding of High Cost Medicines and opinion of the process ?
   - Impact of Medicines Policy upon access to High Cost Medicines ?
   - What improvements could be made in the accessing and funding of High Cost Medicines?

Note: a description of high cost medicines was given, such as; beyond the average person's ability to afford e.g., some oncology and Rare Diseases medicines

6. Supplementary Questions
   - Have the above questions covered Medicines Policy ?
   - Any other aspects of Medicines Policy affecting access, not covered ?
   - Will the current system of medicines access continue, or not ?
   - What is the future for Medicines Policy ?

Anything else to say in relation to Medicines Policy and the accessing of medicines ?

Consolidated criteria for reporting qualitative studies (COREQ): 32-item checklist

Developed from:
Tong A, Sainsbury P, Craig J. Consolidated criteria for reporting qualitative research (COREQ): a 32-item checklist for interviews and focus groups. *International Journal for Quality in Health Care*. 2007. Volume 19, Number 6: pp. 349 – 357

YOU MUST PROVIDE A RESPONSE FOR ALL ITEMS. ENTER N/A IF NOT APPLICABLE

No. Item	Guide questions/description	Reported on Page #
Domain 1: Research team and reflexivity		
Personal Characteristics		
1. Inter viewer/facilitator	Which author/s conducted the inter view or focus group?	Susan Francis
2. Credentials	What were the researcher's credentials? E.g. PhD, MD	RN, PG Dip
3. Occupation	What was their occupation at the time of the study?	Research Assistant
4. Gender	Was the researcher male or female?	Female
5. Experience and training	What experience or training did the researcher have?	Qualitative, NVivo
Relationship with participants		
6. Relationship established	Was a relationship established prior to study commencement?	An email was sent to introduce the objective and scope of the study
7. Participant knowledge of the interviewer	What did the participants know about the researcher? e.g. personal goals, reasons for doing the research	An interest to conduct research on NZ medicines policy issues
8. Interviewer characteristics	What characteristics were reported about the inter viewer/facilitator? e.g. Bias, assumptions, reasons and interests in the research topic	Stakeholder characteristics are described in table 1.
Domain 2: study design		
Theoretical framework		
9. Methodological orientation and Theory	What methodological orientation was stated to underpin the study? e.g. grounded theory, discourse analysis, ethnography, phenomenology, content analysis	Content analysis General inductive approach
Participant selection		
10. Sampling	How were participants selected? e.g. purposive, convenience, consecutive, snowball	Purposive

11. Method of approach	How were participants approached? e.g. face-to-face, telephone, mail, email	Email, face to face, through telephone
12. Sample size	How many participants were in the study?	20
13. Non-participation	How many people refused to participate or dropped out? Reasons?	6
Setting		
14. Setting of data collection	Where was the data collected? e.g. home, clinic, workplace	Workplace, clinic, office
15. Presence of non-participants	Was anyone else present besides the participants and researchers?	No
16. Description of sample	What are the important characteristics of the sample? e.g. demographic data, date	Interviews conducted Dec 2012-March 2013 Fifteen interviews, were conducted face-to-face and five via telephone; due to geographical or time constraints.
Data collection		
17. Interview guide	Were questions, prompts, guides provided by the authors? Was it pilot tested?	No It was pilot tested
18. Repeat interviews	Were repeat inter views carried out? If yes, how many?	No
19. Audio/visual recording	Did the research use audio or visual recording to collect the data?	Audio recording
20. Field notes	Were field notes made during and/or after the inter view or focus group?	Yes
21. Duration	What was the duration of the inter views or focus group?	The median length of interview ranged from 53 – 56 minutes.
22. Data saturation	Was data saturation discussed?	No
23. Transcripts returned	Were transcripts returned to participants for comment and/or correction?	Yes
Domain 3: analysis and findings		
Data analysis		
24. Number of data coders	How many data coders coded the data?	Two
25. Description of the coding tree	Did authors provide a description of the coding tree?	No
26. Derivation of themes	Were themes identified in advance or derived from the data?	Derived from the data
27. Software	What software, if applicable, was used to manage the data?	NVivo
28. Participant checking	Did participants provide feedback on the	No

	findings?	
Reporting		
29. Quotations presented	Were participant quotations presented to illustrate the themes/findings? Was each quotation identified? e.g. participant number	Yes
30. Data and findings consistent	Was there consistency between the data presented and the findings?	Yes
31. Clarity of major themes	Were major themes clearly presented in the findings?	Yes
32. Clarity of minor themes	Is there a description of diverse cases or discussion of minor themes?	Yes (Presented in Table 2)

Once you have completed this checklist, please save a copy and upload it as part of your submission. When requested to do so as part of the upload process, please select the file type: *Checklist*. You will NOT be able to proceed with submission unless the checklist has been uploaded. Please DO NOT include this checklist as part of the main manuscript document. It must be uploaded as a separate file.

BMJ Open

Identifying priority medicines policy issues for New Zealand; a general inductive study.

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Identifying priority medicines policy issues for New Zealand; a general inductive study

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Abstract

Objectives: To identify priority medicines policy issues for New Zealand.

Setting: Stakeholders from a broad range of healthcare and policy institutions including primary,secondary and tertiary care

Participants: Exploratory, semi-structured interviews were conducted with 20 stakeholders, throughout New Zealand.

Primary and secondary outcome measures: The interviews were digitally recorded, transcribed, coded into INVIVO 10, then compared and grouped for similarity of theme. Perceptions, experiences and opinions regarding New Zealand's medicines policy issues were recorded.

Results: A large proportion of stakeholders appeared unaware of New Zealand's (NZ) medicines policy. In general, the policy was considered to offer consistency to guide decision making. In the context of PHARMAC's fixed budget for procuring and subsidising medicines, there was reasonable satisfaction with the range of medicines available – rare disorder medicines being the clear exception. Concerns raised were by whom and how decisions are made and whether desired health outcomes are being measured. Other concerns included inconsistencies in evidence and across health technologies. Despite attempts to enable equitable access to medicines, lower socioeconomic (including rural residents) Māori and Pacific ethnicities and, rare disorders have continued inequitable access based upon need. Other issues related to physical access, convenience to and affordability of prescribers and, the increase of prescription fees from NZ$3 to NZ$5. Concerns related to the Pharmaceutical Management Agency of New Zealand (PHARMAC) included: a constraining budget; non-transparency of in-house analysis; lack of consistency in recommendations between the Pharmacology and Therapeutics Advisory Committee (PTAC). Constraints and inefficiencies in the submission process to access high cost medicines also exist.

Conclusion: The results suggest reasonable satisfaction with the availability of subsidised medicines. However, vulnerable groups; both increasing costs of new medicines and demand; manufacturer order and evidence requirements and, some access procedures still continue to present with issues.

Strength and limitations of the study

Strengths

- This study is the first independent objective study to identify priority medicines policy issues, from a broad range of stakeholders.
- Has identified access to medicines issues requiring further research.
- Provides a context to identified issues.

Limitations

- The views expressed are from 20 stakeholders. Issues raised in this research project are therefore indicative. Further research is required to explore these issues.

Introduction

New Zealand has a population of approximately 4.5 million, with a nominal Gross Domestic Product (GDP) of approximately NZ$211 billion.(1) New Zealanders have an average life expectancy of 81.2 years, which is above the Organisation for Economic Co-operation and Development (OECD) countries average of 80.1years.(2) Just under 83 percent (82.7%) of health expenditure in New Zealand is publicly funded.(3) New Zealand's health and disability budget at the commencement of this research is NZ$13.983 billion.(3) In comparison to other OECD countries, as a percentage of total expenditure on health, New Zealand spends less on pharmaceuticals.(2) Approximately NZ$795 million and NZ$280 million are available, for procuring community/cancer and hospital pharmaceuticals respectively.(4) This compares with a reported estimated spend of NZ$880 million on medical devices.(5) Approximately 1848 medicines are subsidised by PHARMAC (as listed on its Pharmaceutical Schedule) for use in the communityand are largely accessible via prescription from a medical doctor.(6)

Medicines and New Zealand

Medicines make a significant contribution to health outcomes.(7) In 2007, "Medicines New Zealand" New Zealand's medicines policy, was launched in response to access concerns from the public.(7) The aim of the policy is to promote quality, effective and optimally used medicines. To guide decisions, principles of affordability, equity and need are stated.(7) Medicines New Zealand aims to ensure that the decisions made about prioritisation and funding are as transparent as possible, understood and open to debate. It is important for New Zealanders to have confidence that the medicines system is fair, even if they do not always agree with the decisions made (7).

Pharmaceutical Management Agency (PHARMAC)

PHARMAC, established in 1993 in response to increasing expenditure on pharmaceuticals, is a separate non-profit government agency, whose role is to determine and procure, community and oncology medicines on behalf of New Zealand's 20 District Health Boards (DHBs). PHARMAC has a pre-determined fixed budget which it is required to operate within. In order to provide medicines considered necessary, PHARMAC employ therapeutic and economic analyses to guide decisions. PHARMAC's scope is now expanding to include hospital medicines and some medical devices.

For the majority of patients prescribed a medicine listed on PHARMAC's schedule, a NZ$5 District Health Board charge is incurred. For high user or low socioeconomic patients, access enablers(e.g., Prescription Subsidy, Community Services and High User cards and, recently the Services to Improve Access (SIA)) exist to help ease financial burden.(6)

Literature exist indicating medicines issues for New Zealand related to: inequities in access, affordability, processes used and their funding. (8-31) However, no systematic work has been conducted to identify priority medicines policy issues with regards to access and funding of medicines. Within this context, it was considered timely and appropriate to conduct research that could identify priority medicines policy issues for New Zealand.

The dataset obtained from this research was expected to be substantial and provide a solid platform to contribute towards informing medicines policy, expenditure and provision and, the development of optimal medicines management strategies.

Aim

The aim of this project was to identify priority medicines policy issues for New Zealand.

Methods

Study Design and Participant Selection

We conducted a general inductive study, using semi-structured exploratory interviews during December 2012-March 2013. Selection was purposeful, to ensure a broad representation of stakeholders and their opinions, who had one or more of the following traits in relation to medicines policy: involvement in its formation or implementation, had researched and/or commented on medicines policy, including having made submissions during its development, (n = 10); medically qualified doctor (n = 7, 4 of whom were active prescribers, including one of each of Māori, Indian and Asian ethnicity); medicines regulation (n = 1); representation of or, past or current involvement in medicines supply, procurement, funding or provision (excluding dispensing, n = 6); involved in medicines management (n = 9, one of whom was of Māori ethnicity); medical information or health technology assessment interest (n = 2); medical interest group representative (includes ethnic medical group/association, past or present, n=4); private health provision and subsidy (n = 1); patient group representative (n=2, one of whom represents and advocates for a large chronic disease group, disproportionately represented by Māori, Pacifica and increasingly the Indian and Asian ethnicity). The participants characteristics are summarised in table 1.

A total of 26 stakeholders were contacted and explained the research involvement. Twenty stakeholders consented and interviewed. All 20 received a "Participants Information" letter, detailing the involvement, aim and general methods. All signed a confidentiality and anonymity agreement. Fifteen interviews, were conducted face-to-face and five via telephone, due to geographical or time constraints. The average length of interview ranged from 53 – 56 minutes. No gratuity was offered.

Instrument development

The main aim of this research was to identify priority medicines policy areas. An in-depth literature review was conducted, to ascertain existing information on pharmaceutical policy. A total of 105 references were identified as useful. The following broad themes were discovered and accordingly, sets of questions developed: (1) Medicines Policy: including participant's awareness, description and opinions; (2) Ethnicity inequities in accessing medicines (viz Māori, Pacifica and recently immigrated people whose first language was not English); (3) PHARMAC: its pricing policy, impact upon access, economic modelling, performance, future and any improvements; (4) The Trans Pacific Partnership Agreement (TPPA): impact upon access and resultant considerations; (5) High cost medicines access; (6) Medicines policy issues not covered but considered important (see appendix one for question details)

The questions were piloted on one doctor of Māori ethnicity and one pharmacist (Ph) with an interest in medicines policy, medicines management and academia, who has previous experience in the pharmaceutical industry. Their responses were not included for analysis.

Data Collection

Participants (stakeholders) were encouraged to give comprehensive answers. Clarifying and confirming questions were asked where more information was considered necessary, or to avoid interviewer assumption.

All interviews were recorded on a voice recorder, transcribed intelligently (space fillers were omitted to enable ease of reading) Participants received their own transcript to proof, edit and approve. Only the approved editions were entered into INVIVO 10 (QSR International Pty Ltd) for coding.

Coding was conducted two ways: firstly, categorically according to answers and secondly, highlighted, grouped and compared according to similarity of theme. Transcripts were checked for any missed issues.

A check for stakeholder bias was conducted using the coding summaries, no apparent bias was detected. Any variations appeared attributable to stakeholder knowledge.

Results

Issues revealed specific to ethnicity, PHARMAC, the TPPA or high cost medicines are reported in those sections. A summary of issues is available in table 2.

General Medicines Policy Issues

Nine participants stated they were unfamiliar with the policy. However, four demonstrated a tacit understanding. It was questioned how policy intentions and decisions are made, in the context of being achievable:

> *"How do you attain that?...what is the right way to make those overall policy decisions…" (pharmaceutical industry stakeholder, PI)*

All participants believed medicines make a positive contribution to health. Differing levels of impact upon health were noted. There was uncertainty as to how the impact is, or could be quantified. The lost opportunity from not capturing and accessing data efficiently, was voiced by two academics for both treatment and outcomes monitoring:

> *"…we are not asking questions about patient health status before and after… so you can really see what is going on, at the GP level. Because that's at least as important as hospitalisation data". (Academic, Ac)*

Conversely, one participant said he would prefer to see more investment in epidemiology, as opposed to increasing the medicines budget, in a desire to preserve health.

Low socioeconomic patients were considered to have a higher burden of disease. Affordability to prescribers was described as the major issue, which may be compounded by the 2013 raise in prescription co-payment from NZ$3 to NZ$5.

Despite access enablers, such as the High User Cards and Community Services Cards, it was questioned whether those in need are utilising them. One General Practitioner (GP) said cost-sensitive patients could be managed with prudent prescribing and education on priorities:

> *"You could get all your medicines for less than a pack of cigarettes. It's educational priorities and various other things, where the effort needs to go rather than reducing the cost much further." (GP)*

The opposite situation of the misuse of access was described:

> *"I initially struggled to understand how somebody could pull up outside a pharmacy in a Mercedes Benz and… present their scripts for their family and handover their Community Services Card… As soon as they get in the country; they put the money into a family trust...So the wealth of the individual gets assessed, which qualifies them for a Community Services Card and then they wave that around." (Pharmacist, Ph)*

Sole supply[1] provision raised issues in terms of: supply outages when switching supplier (and having to pay for the alternative option) options for patient intolerance and vulnerability if a significant disruption in supply occurs (e.g., a disaster destroying a supplier's warehouse).

There was additional reference to policies and funding needing to be consistent and interlink, especially for priority areas. "Quit-Line" was given as an example: a $40 million funded smoking cessation programme, described as having markedly less evidence than the appropriate medicines (which were not funded for many years). Budgetary constraints were the reason given for this. Equally, the government funding of "alternative medicines" was described as something which needed debate:

> "*… government is providing funding for people to obtain alternative medicines … real debate to be had… money better spent some place else in the healthcare system?*" *(Pharmceutical Industry, PI)*

One Doctor voiced frustration at PHARMAC's Therapeutic Advisory Committee (PTAC) [2] being *"generalists"* who over-ride the recommendation of their subcommittee. With patient sub-typing and genomic medicine on the horizon, he considered *"generalists"* may not understand what they are assessing and dismiss research, thereby inhibiting access. Access is then through an ability to pay for litigation and decided by a non-medical expert.

With demographic changes increasing demand for healthcare services and a general movement towards increasing costs of new medicines, there was concern for future affordability of medicines. It was suggested funding may move away from being population basedand move toward funding health outcomes. An academic suggested changes in co-payments, taxation or medicines classification status may result. The oncologist had concerns that the lack of research (research being an attracter) being conducted within New Zealand, will compound the low availability of future medical specialist prescribers. Extending prescribing ability to non-doctors was considered to help. However, for oncology, a medical specialist was still considered to be required to make treatment decisions.

Ethnicity Issues

Most issues presented, related to socio-economic variables and are presented under General Medicines Policy Issues.

Those with poor English speaking skills were described as having access to an English speaking relative or even interpreters if needed:

[1] Sole supply arrangements are likely to be used by PHARMAC in markets where generic competition exists, resulting in there being only one brand of a particular chemical listed. It is possible that PHARMAC would agree preferred supplier status for some chemicals in exchange for price concessions, affecting access to related pharmaceuticals within the same therapeutic group.Pharmaceutical Management Agency. Proposed pricing strategy initiatives - sole supply arrangements. Pharmaceutical Management Agency; 2002 [cited]. Available from: http://www.pharmac.govt.nz/2002/07/19/nhps.pdf.

[2] PTAC is PHARMAC's primary clinical advisory committee. PTAC's role is to provide clinical advice to the Board of PHARMAC.

> *"I think if they can access General Practice or the Hospital system, their access to the medications is just as good as anybody else's. I'm not aware of any specific ethnic problems in accessing our medicines.." (GP)*

One GP felt strongly that Māori and Pacifica access inequities are evident and are reuslting in poorer health outcomes. He considers his colleagues are treating everyone the same but with inequitable risk: earlier intervention, improved communication, education and patient engagement are required:

> *"I think the key issue is the prescribers have a poor understanding of inequalities. Because, the prescribers generally approach things as; I treat everyone the same… they must have an inequity lens on anyone they see… but if the quality of your discussion and the quality in the way in which you prescribed that was poor i.e., you culturally are incompetent and you have a disconnect with the patient…" (GP)*

Other issues related to Asian ethnicities wanting treatment (oncology setting) irrespective of likely outcomes and the use of alternative treatments e.g., St John's Wort or Vitamin C injections impacting upon medical treatment. One of the doctors had issue with alternative practitioners recommending such treatments.

Pharmaceutical Management Agency (PHARMAC)

There was general appreciation shown towards PHARMAC's strategy of creating competition in order to achieve a lower purchasing price. This was seen as advantageous for the purchasing of a greater range of medicines, in the context of a fixed budget.

The budget was defined as the threshold for provision, which was considered too small by an academic and pharmaceutical industry stakeholder , causing a focus on cost as the driver of value and provision, thereby contributing toward *"static efficiency"*:

> *"If Pharmac's objective is to stay within budget then it's doing well… improve the health of New Zealanders within a capped pharmaceuticals budget…it's doing moderately well…objective were to improve the health of New Zealanders taking into account the financial constraints of Vote Health…it's doing poorly because it should be fighting for a better share of Vote Health." (Ac)*

A public service stakeholder (PS) offered the following standpoint: *"You can always achieve more with more." (*in terms of a bigger budget) but an analytical framework is not in place that would define whether the medicines budget receives a fair proportion of "Vote Health" or not.

PHARMAC's budget is determined at regular defined intervals but medicines enter the marketplace sporadically. This caused concern for the oncologist, that extra funds may not be available to deal with this.

The private health care organisation stakeholder (PHCO) thought PHARMAC's approach of requiring new and more expensive medicines to be better than standard medicines a *"completely acceptable approach."* It was suggested by the PHCO and an academic that their approach could be more widely adopted, both overseas and with the expansion of PHARMAC's role to medical devices:

"…the expertise PHARMAC has built up…is something that we could learn from and borrow from, for the wider health sector… I'd like to see them take on medical devices, because that is absolutely scandalous that these products are getting onto the market without being properly evaluated…" (Ac)

There was caution given from one pharmacist that PHARMAC's expansion into hospital medicines (in an acute care setting of moribund disease) may limit choices. Concern was shown for risk, if New Zealand is world-leading in this type of provision.

Provision was described as having a utilitarian focus: *"The greatest good for the greatest number" (PS)* and described as being: you get what you need - not what you want. One doctor questioned whether the lost opportunity from not treating someone is being measured. Rare disorder patients were mentioned and are discussed under High Cost Medicines. The distinction was made that provision of a medicine in a cost effective manner, which PHARMAC achieves, is not the same as delivering healthcare:

> *"I find some of their PR a little bit irritating…bray on about the marvellous healthcare they're delivering… delivering medicines in a cost effective manner but that's not saying it's delivering healthcare…" (PHCO)*

Questioning PHARMAC's undertaking of economic modelling , received very favourable comments from 15 of the participants. Three participants were not familiar with economic modelling:

> *"I think it's world leading actually. No one else dares do it. That's the crazy thing. Here we are little old New Zealand and we dare do it." (Ac);*
>
> *"Well I mean, as a tax payer you could argue that for the majority of the products they get in, they've done a really good job of driving cost out of the system." (PI);*
>
> *"Technically it's very good. PHARMAC considers clinical effectiveness and cost effectiveness.. they make trade-offs… they look at the QALYs and the number of people affected and how their quality of life will be improved and so on, I think is a very good model." (PS)*

It was suggested that the cost-benefit of returning an individual back to their normal daily activities should be considered by PHARMAC, such as what Accident Compensation Corporation (ACC) does when assessing intervention options.

A pharmaceutical industry stakeholder and academic were concerned the required economic modelling submitted by suppliers is adjusted with unknown *"in-house"* variables, making it hard for suppliers to understand decisions. This was contrasted against Medsafe's practice, where decision modelling is transparent:

> *"Pharmac receives a dossier from the company…Assumptions of statistical models get changed…QALYs get changed…population who will use the product get changed…that should be part of a scientific debate…companies don't know what information is being used to make the decisions on their products…we would like a right of reply to those… It happens with MedSafe…Not as though it could potentially negatively affect evidence based decisions."(PI)*

Delays in the submission process of up to eight years and described as a *"medicines waiting list,"* were of concern for an academic, pharmaceutical industry stakeholder and patient group representative (PGR), who all thought access was related to cost. There was suggestion from one academic to follow Australia's submission process and out-source assessments from independent bodies.

Trans Pacific Partnership Agreement (TPPA)

Very few participants were familiar with the TPPA, some referred to speculation and no facts. One participant refused to give any comments related to the TPPA.

There was acknowledgement that trade deals are complex and often require compromises and trade-offs. New Zealand was referred to as a *"small country"* and *"we need our trade partners."* There was concern that already "*big amounts"* are being spent on healthcare and the *"benefits are low"* and, if there is a resultant increase in the cost of medicines, where would resources come from, to off-set any cost increases.

The main issues were: (1) Patent extension, delaying generic entry to market, thereby prolonging a higher cost of provision; (2) Industry influencing supply (described as an issue of sovereignty) may result in quicker access to new medicines but also an increase in public campaigns and appeals processes if PHARMAC's decisions are unpopular with the pharmaceutical industry or patient groups; and, (3) Transparency requests in PHARMAC's assessment process caused the most concern and confusion. One academic said he didn't think PHARMAC could be more transparent and that transparency might mean the industry discloses its pricing processes and the results of all clinical trials.

In general, scepticism was voiced as to what the driving force is behind the agreement and what the benefits would be for New Zealand - with the United States of America (USA) being a protected market (heavily subsidised). Australia was described by a pharmaceutical industry representative as getting *"trounced"* over their agreement with the USA, losing a lot of their pharmaceutical production and jobs as a result:

> *"Forget it…wouldn't even bother going along to the negotiations"(PHCO)*;
>
> *"…tell the US to bugger off quite frankly. You either put everything on the table and we talk about it or no, you don't…We should learn from what happened in Australia…" (PI)*

Conversely, another participant suggested whilst *"America"* has influence, it may become limited as a result of the influence of China's developing economy and differing ideas around protection and, new opportunities may develop:

> *"…a hugely developing economy in the form of China that basically has total disregard for such things… so the ability for America…is probably going to be limited in the world of the future, and maybe different forms of protection of ideas will kind of evolve… it's very hard to predict how the market might respond or what kind of new opportunities develop." (PS)*

A pharmaceutical industry stakeholder said Medicines New Zealand (New Zealand's prescription medicines representative association, same title as the policy) is attempting to ensure its United States of America equivalent understands New Zealand's medicines system:

> "*…working quite hard to ensure…our sister organisation in the US is effectively asking the US government to achieve out of the process, is well enough informed to understand actually what the New Zealand model does achieve, what it doesn't achieve and how that can be improved…So we're working hard to make sure it's a process that actually benefits New Zealanders as well, and all of the transparency, timeliness, appeals – those aspects that we've discussed, are exactly I think what the US is likely to be asking for." (PI)*

Most considered that New Zealand's current ability to access generic medicines or, independence in procuring medicines should be upheld. If not, funds may need to be redirected from other services or, patient co-payments would need to rise, in order to compensate a likely increase in the cost of medicines.

High Cost Medicines

A GP questioned the necessity of continuing the Special Authority (SA)[3] status for a medicine, once the appropriate use of a medicine has been established. Not all participants were familiar with the Named Patient Pharmaceutical Assessment (NPPA)[4] access scheme. Most high cost medicines were described as being *"breakthrough"* or *"expensive"* and are restricted, to control spending. One participant said, if it was *"dirt cheap,"* there would be "*no argument,"* indicating the case even if the medicine didn't have clear health benefits:

> *"My bet, is that PHARMAC would listen to anyone that agrees with them saying no. Because it's expensive ....They are diametrically opposed for a reason and the reason is cost." (Ph);*
>
> *"The Rabbits in charge of the lettuce patch" (M)*

One public service stakeholder thought there to be no inappropriate blocking of access to medicines, as no complaints about access have been received at their level. Equally, another public service stakeholder commented that there are patients accessing medication costing up to NZ$500, 000 per year:

> *"…So it's not that the system can't cope with treatments that are high cost, it's just that we would expect a return for that cost and for it to be justifiable in terms of what we value." (PS)*

A small group of patients were described as not having access to high cost medicines. Access was described as *"the collective good."* Conversely, *"people dying from a lack of access to very cheap and simple therapies"* was described. It was suggested that it is a DHB's remit to look after its population, highlighting the issue of population versus individual access. An assertion was made, if are we advocating treatment at any cost, who pays:

> *"It's a question of who pays for all these things. I think if you have pretence; like there is in the USA, that cost isn't of any relevance… then you're going down the wrong path." (PHCP)*

The oncologist described the NPPA process as inefficient, a comprehensive and referenced application, takes him up to six hours, potentially impacting on his clinic time and perversely hindering patient access. He suggested PHARMAC at a nominal cost could employ someone to aid in information gathering and in the process develop expertise.

Additionally, the oncologist believes oncology has the stigma that everyone dies but individual survival may be greater than the median survival assessment. This issue was presented in comparing the availability of two to three drugs in Australia that are unavailable in New Zealand.

Questions were posed: (1) Is it fair to give fourth or fifth line chemotherapy and not give a first line treatment e.g. for rare disorders? and, (2) When do you stop treatment, a patient was described as gaining access to expensive medication, their condition was fragile and they died a few weeks later:

> *"I think it's important if Pharmac has a few loose strings in terms of hospital and severe rare conditions. They are perhaps because of how they are funded, they want a very narrow perspective*

[3] Special Authority criteria define the clinical circumstances of patients who can receive funding for the medicine. People may first be required to try a less expensive medicine or the medicine may need to be prescribed by a particular type of health practitioner.

[4] NPPA is a mechanism to give individual named patients access to medicines they need, but which aren't funded on the Pharmaceutical Schedule. NPPA replaces the three Exceptional Circumstances (EC) schemes that PHARMAC previously managed.

on those, to try and avoid blow out. They are very emotive issues we don't always know how to best manage people's care. " (Ph)

High need patients, such as rare disorder patients received the most sympathy for difficulty in access because of the exclusion criteria. Evidence requirements were described as difficult to attain due to low patient numbers. Conversely, the PHCO suggested the supplier needs to produce quality evidence:

"...where there's some evidence, but not solid or quality evidence: ... the company doing the – providing the medication, it behoves on them to do some research in those areas and produce quality data."(PHCO)

Discussion

We purposefully attempted to be open to issues and their capture, despite some issues already being identified. Our focus was on access to medicines. It is possible there are other issues in existence, we neither recognised, nor captured. We did not seek to determine issues specifically related to generic medicines, considered a "*vital component of New Zealand's medicine cost management policies*" by Babar et al.(12)

Medicines Policy

Medicines are clearly valued health interventions: evidenced by the budget, literature and responses from stakeholders. The smaller percentage spend on pharmaceuticals in New Zealand (described as a constrained budget) compared with similar countries such as Australia, the United Kingdom (UK) and the USA,(2) may in fact reflect the price reduction strategies that are implemented by PHARMAC(32), as opposed to less opportunity to improve health outcomes. However, this needs to be tested through robust research on health outcomes and their relationship to pharmaceutical spending.

Delayed access and the resultant impact discussed by some of the participants, was also described by Ellis and Hamer,(33) in relation to New Zealand's statin availability for atherosclerotic patients, as probably negatively impacting health outcome and considered to be due to the capped budget. They considered this *"anomalous,"* as other types of health care are not capped. This anomaly was also described by a number of participants but may change with PHARMAC's expanding role.

New medicines are increasing in costs along with demand, causing tension in affordability. Price efficiency initiatives, such as what PHARMAC encourages, help ease the tension in affordability of provision. Another option is to reduce demand, either through "gate keeping" (not usually a popular choice) or genuine effects, such as initiatives to maintain health or prevent disease. We assume a reduction in demand and therefore burden of provision, should result in healthcare becoming more affordable, for providers and helping those remaining in need.

Manning,(34) compared the processes of decisions, pricing, economic analysis, provision and access and, participation and appeals between the UK, Australia and New Zealand. It was suggested that resolving issues may benefit from a disputes panel comprising a broad range of experts in scientific, economic, policy and ethical evaluations, in order to provide an objective decision. Manning additionally reported that approximately one sixth of the United Kingdom's National Institute for Healthcare and Clinical Excellence (NICE) recommendations are appealed and upheld.(35) There shouldn't be great demand, if evaluation processes are robust.

Ethnicity

Our participants revealed that low socioeconomically related populations (encompassing Māori and Pacifica people) are continuing to have access issues related to financial, structural, educational and cultural barriers. These findings were consistent with that of Jatrana et al,(36) who assessed SOFIE-health's 18320 respondents (an add-on to Statistics New Zealand's longitudinal survey of Family, Income and Employment). Māori and Pacific people were more likely to defer purchasing a prescription due to cost, which at that time was NZ$15.

Māori represent approximately 15 percent of New Zealand's population(37) and on average have the poorest health status of any ethnic group in New Zealand.(38, 39) Pacifica people represent 6.5 percent of the population and also experience health inequalities. He Korowai Oranga: the Māori Health Strategy (2002),(38) recognises the Treaty of Waitangi principles of: partnership, participation and protection, through which, the aim is to reduce existing health inequalities. This aim is extended to include the Pacific people, who like Māori, they are over represented by a low socioeconomic situation, reflecting low affordability and health literacy, which in turn affects access. The Ministry of Health recently launched Services to Improve Access(SIA)(40) an additional targeted capitation payment, available to primary healthcare organisations(PHOs) to reduce health inequalities. It is designed for new services (e.g., outreach programmes) or improving access (e.g., funding transport) for Māori, Pacific people and those of low socioeconomic status. Once SIA is embedded, it would be prudent to evaluate its impact.

Other Ministry of Health initiatives, such as Whanau Ora (to build the health, participation and capability of families) and One Heart Many Lives (to improve the cardiac health of Māori and Pacific men) along with recent changes in health practitioner training, appear good initiatives for engaging Māori and Pacifica in a culturally appropriate way. It would be prudent to evaluate their impact.

We did not have any issues specifically described for new immigrants. It was described to us that patients with poor English speaking capability, present to practitioners with an English speaking person, or frequent a surgery of their ethnicity. This is at odds with Babar et al,(9) who found for 11 Chinese and Indian migrants, residing in New Zealand for less than five years, financial barriers existed in affording doctors, pharmacists and medicines and, that language barriers exist. This anomaly may highlight the differences in both the perspective and experience of the stakeholders we interviewed.

Asia and India have different medicines access systems to New Zealand. Babar et al additionally found there is a lack of information on New Zealand's medicines system, including medicines provision and classification. The United Nations and World Health Organisations, when discussing the right to health,(41) refer to migrants as being vulnerable to reduced access to health services for reasons that include language or cultural barriers. New Zealand has a significant migrant population, reported as 927 000 in 2006.(42) The current main countries for immigration are: China (15%), United Kingdom (unspecified), India (13%) and the Philippines (8%).(42) In consideration of Babar et al's work and immigration statistics, there may be a significant number of people from these countries with issues, resulting in difficulties in accessing healthcare and therefore medicines.(8-10) In light of this, it may be worth further investigating new immigrant issues.

PHARMAC

The general appreciation for New Zealand needing to be efficient, in order to provide more medicines, expressed by our stakeholders, was also shown by Ragupathy et al.(26) Included, was the need to apply consistent economic evaluations to other health technologies, to support congruous decisions for resource

allocation. PHARMAC's expansion into procuring hospital medicines and medical devices may enable greater consistency of evaluation across technologies.

The significance of PHARMAC's role expansion should not be underestimated. PHARMAC will need to practice caution with expanding their role into hospitals, which are generally settings of acute and moribund disease. We are unaware whether a closed formulary has occurred elsewhere in the world. PHARMAC have been noted to have consulted directly with medical specialist groups to discuss their role expansion, including consultation on hospital medical devices,(43) so would appear to be fully cognisant and appreciative of this issue.

We found delays of up to eight years, in PHARMAC's process for funding medicines onto the Pharmaceutical Schedule, which a number of our participants purported to be due to the medicine's pricing and/or PHARMAC's budget not being able to expand. Other reasons may be the medicine's priority status, insufficient information or, not meeting PHARMAC's nine decision criteria.(44) The question is whether this means delays in therapeutic advancement and therefore improved health outcomes.

The measurement of opportunity foregone was of clear concern to the oncologist we interviewed. New Zealand has a capped medicines budget, it cannot expand and therefore drives the need for efficient spending (determined using cost utility analysis, where medicines are assessed against QALY gains per NZ$1 million) Using this process for provision means there is opportunity foregone, as described by Milne and Wonder.(14) We are not aware of New Zealand focused research assessing either opportunities foregone or, other specialist viewpoints on access. The exceptions being: Ellis and Hamer(33) in 2008, discussing the delayed availability of cardiac medicines; MacCormack et al in 2009,(31) assessing stakeholders views on needed access to high cost medicines and; The Sage report for the Ministry of Health in 2010, (22) reporting the consultation of stakeholders on the proposal to expand PHARMAC's role.

Sole supply issues (supply outages, lack of palatable formulations, resultant out of pocket payments for alternatives and vulnerability as a result of a disaster) were reported as still continuing, despite there being penalties for suppliers. This was also reported by Babar et al,(45) who reported additional concerns with poor quality products in the past, from previous studies.

Trans-Pacific Partnership Agreement (TPPA)

There is very little information available on the TPPA, the reason given: particulars of the negotiation are changing. What does exist concurs with our findings, it questions the motivation and self-interest of parties involved and warns of possibly binding impacts that may affect health services budgets and PHARMAC's autonomy, including method of procurement and provision.(25-29, 46) Such impacts stem from the USA's desire for stricter protection of intellectual property rights, transparency of in-house evaluation, regulatory coherence, dispute settlement, government procurement and evidence based decisions being contestable in court. Unless budgets expand to cope with likely increases in costs, there may need to be a re-evaluation of provision, subsidies and co-payments. In contrast to existing publications, our research additionally suggested a TPPA may enable earlier access to newer medicines. It may be of use to quantify what effect a TPPA would have upon medicines access.

High Cost Medicines

A significant issue discussed in our study, was the need to differentiate between high cost medicines and both highly specialised needs and medicines in relation to the NPPA access scheme. McCormack et al, (31) suggest a medicine that costs NZ$20 000 per patient per year may be considered high cost. It is important to be cognisant of the total cost to the health system of any medicine, which is dependent upon the number of patients treated (volume used) and the acquisition price. Some high cost medicines may not result in a high total cost to PHARMAC, for some patient groups. Gallego et al,(47) question how treating large populations at high total cost for small population gains, compares with treating smaller populations, for possibly significant benefit.

The issue of treating large versus small populations may intensify with patient subtyping and genomic medicines development (as described by the oncologist) where greater expectation to fund (i.e., demand) may occur. With the NPPA process now reported in our findings, as enabling the capturing of cancer patient subtype information, cancer medicines outcomes may become easier to measure and if positive, make it harder to decline funding treatments. It may also mean the table is turned and large populations end up having limited treatment options, if outcomes cannot be measured in the same way. However, funding outcomes will give a clear indication for innovation and direction to both suppliers and funders of medicines.

Our findings describe both the SA and the NPPA access schemes as being inefficient. The SA inefficiency finding is also supported by Babar et al's evaluation of GP perceptions on access to medicines in New Zealand.(45) Once correct prescribing of a medicine has been established it may not be necessary to continue a medicine's SA status. The NPPA process appears to impact significantly on consultant clinic time which may perversely hinder patient access. With demographic trends indicating greater demand for such medicines, the impact of the inefficiency may intensify. PHARMAC's website lists 555 approvals and 15 declines for NPPA access.(48) The high rate of NPPA approvals, brings into question the need for such a process. Alternatively, it may be more efficient for PHARMAC, at a nominal cost, to contract an evidence based facilitator, to ease the burden of application for clinicians.

Difficulty in access to high cost medicines, encompassing rare disorder patients, as described by our participants, has been widely documented.(31, 47, 49-58) The nature of rare diseases makes it hard to gain the necessary evidence PHARMAC requires for evaluation. This issue is compounded for suppliers because the need to satisfy both manufacturer ordering and regulatory requirements, adds to the unit cost of supply for low volume demand medicines. It may be worth investigating options to reduce cost of supply and provision in the context of constrained evidence. PHARMAC have recently sought public and professional input into its decision criteria. The results have yet to be published but may reveal new options or initiatives.

Our research highlighted the issue of access to medicines of therapeutic value in the context of a fixed predetermined budget and the difficulties in how priorities for funding are determined. Lu et al,(59) in discussing ethical perspectives to the access of high cost medicines in Australia, discussed the issue of having equal need requires equal opportunity to access care and suggest where evidence requirements are not achieved, treatment commence on a trial and outcome basis. This does come with ethical concerns but may enable both access and capturing evidence. MacCormack et al,(31) suggest "risk sharing" supply to ensure some form of access (defining a threshold for maximum numbers to treat for a high cost medicine, above which, the supplier funds)

Conversely, Simoens et al,(52) caution providing access to medicines with limited effectiveness, implies rare disorders health improvement is more valuable than a common disease, which challenges the utilitarian view of: the health gain of each patient is valued equally. With both increasing effort in the development and availability of orphan drugs, this issue may only worsen. Equally, other questions arise: because we see the

ill health, can't mean preferential treatment over someone who has a "silent" state of declining health. There are people not getting access to inexpensive medicines, who are at risk, as stated by an academic. Perhaps remedying issues of access based on need, could start with prioritising based on the impact of an unmet need?

Conclusion

Overall, despite issues being identified, there was reasonable satisfaction with the New Zealand's medicines policy and its principles. In particular that provision is evidence based, cost effective and there is equitable ability to have prescribed medicines listed as subsidised, on PHARMAC's schedule.

However, despite this, there appears to be some patient groups still experiencing difficulties in access, not necessarily appearing as a result of medicines policy or PHARMAC. Such groups being rare disorders and the low socio economic (encompassing rural, Māori and Pacifica populations). Other issues ranged from: the pharmaceutical industry's pricing of new medicines; manufacturer and registration requirements; the submission for funding process; increasing demand for medicines and the resultant financial impact; budgetary constraints; cultural and health literacy; patient affordability and access to prescribers; through to knowledge development for clinical expertise and the measurement of health outcomes.

Our study has highlighted issues in access based upon need and the consequences of unmet need. The context being a fixed and predetermined budget, increasing demand and the rising cost of medicines are all compounding constraints in affordability. We suggest these issues and consequences of unmet needs may worsen and options for demand and provision may need to be explored further.

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Ethics: Ethics approval was obtained from The University of Auckland, Ethics Committee. Approval number; 8367

Authors contribution: ZB conceptualised and designed the study. The data collection, entry and analysis was handled by SF and ZB. SF and ZB wrote the manuscript. The final version is approved by all authors. ZB acts as an overall guarantor to this study.

Transparency: ZB affirms that the manuscript is an honest, accurate, and transparent account of the study being reported; that no important aspects of the study have been omitted.

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Data sharing statement: The original data are available from the principal author (ZB)

Table 1: Stakeholder Characteristics:

Stakeholders n = 20

STAKEHOLDER	NUMBER	COMMENTS
Academia (A)	3	Sociologists (2) Pharmacoeconomist (1)
Public Service (PS)	5	Politician (1) Medsafe (1) Policy Analyst (1) DHB Planning (1) Pharmac (1)
Medicine (M)	4	Oncologist (1) General Practice (3)
Pharmacist (Ph)	3	DHB (2) Community (1)
Pharmaceutical Industry (PI)	2	Manufacturing (1) Representative (1)
Patient Group Representative (PGR)	2	Long Term Conditions (2)
Private Health Care Organisation (PHCO)	1	Medical doctor (1)
Additional Attributes of Stakeholders		
• 3 Medically qualified Doctors		• 1 Health Professional of Māori ethnicity
• 1 Pharmacist		• 4 Doctors past or current Medical Group Representation
• 3 Scientists		• 3 GPs ethnicities; Māori, Indian and Asian; past or currently practicing in areas with high numbers of respective ethnicity, including Pacifica
• 1 Medical IT		
• 1 Therapeutic Assessor		

Table 2: Summary of issues

General Medicines Policy	Ethnicity	PHARMAC	TPP	HCM
• By whom and how are decisions made? • Poor Medicines Policy awareness • Poor Health Literacy; impacting timing of presentation and medicines adherence • Access to Prescribers; physical, timing and affordability • Socio-economic factors (encompassing rural residents) • Sole Supply; out of stock vulnerability and cost, options for intolerance • Discord in recommendations between PTAC and subcommittees • Access challenges on the ability to pay for litigation; non-medical person then decides access • Lack of health impact monitoring • Need for integrated electronic patient records, prescribing information & PHARMAC schedule • Efficiency is static; needs to move toward increases in therapeutic benefit • Registration, evidence and manufacturing requirements constraining for low demand medicines • Increasing demand and cost of medicines impacting affordability • Need for clinical expertise and New Zealand specific research • Need for better medicines management	• Socioeconomic factors • Need to use "Health Equity Assessment Tool" to assess policy & inequities/inequalities • Higher burden of disease for Māori and Pacifica; needing risk factor lens • Lacking proper engagement at times • Cultural competency • Use of alternative medicine • Need to capture ethnicity statistics in new initiatives	• Very powerful position of provision; will they cope with role expansion • Young inexperienced staff and high attrition rate • What health outcomes are being measured • Is the lost health opportunity being measured? • Budget too small; need higher percentage of Health budget; "Vote Health" • Cost driving value & causing delays • Need to move to dynamic efficiency • Need analytical framework to compare all health technologies • Submission process inefficient • Economic evaluation influencing therapeutic value evaluation; need to be separated • Questionable how well health professionals understand pharmacoeconomic modelling • In-house economic variables are not necessarily consistent with standard practice or PHARMAC's requirements of suppliers • Hard for suppliers to understand outcome or evaluation process when variables changed • Website very informative but hard to navigate • Concern with expansion into hospitals & limiting choice in acute care & moribund disease setting • Sustainability of current access with increasing demand • Affordability of a panacea	• Many unfamiliar and sceptical of the benefits and who gets them vs. the trade offs • New Zealand small country that needs trade partners • Where will the financial cost be felt and how will it be dealt with • Will there be an increase in the cost of provision • A lot money being spent on health already and benefits low • Australia lost a lot with their agreement with USA; we should learn from it • America's influence is reducing and other forms or protection may evolve • Patent extensions will delay generic entry and raise costs • Will the pharmaceutical industry have greater influence on supply • Access to new medicines may improve • Sovereignty of choice; will there be increased public appeals & litigation • What does transparency mean and does it "cut both ways" • PHARMAC's monopsony is an anathema to the USA • NZ pharmaceutical representative educating "sister" organisation in NZ system • Once a medicine is registered for use, it can be prescribed; PHARMAC may choose to not fund it	• Special Authority access unnecessary once appropriate prescribing established • Need to differentiate high cost vs. highly specialised need and cost • NPPA access scheme brings equitable access for oncology but too early to assess • NPPA capturing patient sub-type classification • NPPA process inefficient and consuming valuable specialist time • Limits access due to cost; but about collective good and who pays • Access cheaper in other countries? • Pharmaceutical companies have good profit margins • Oncology stigma that everyone dies but differences in survival seen at the margins • Evidence does not meet PHARMAC's evaluation criteria • Constraints of "rule of rescue" vs. utilitarian provision

References

1. Statistics New Zealand. Top Statistics. 2013 [cited]. Available from: http://www.stats.govt.nz/browse_for_stats/snapshots-of-nz/top-statistics.aspx.
2. Organisation for Economic Co-operation and Development countries. OECD Health Data 2013 - frequently requested data: health expenditure. 2013 [cited]. Available from: http://www.oecd.org/els/health-systems/oecdhealthdata2013-frequentlyrequesteddata.htm.
3. Ministry of Health. Funding. Ministry of Health; 2013 [cited 2013]. Available from: http://www.health.govt.nz/new-zealand-health-system/funding.
4. Pharmaceutical Management Agency. Setting and managaing the combined pharmaceutical budget (CPB). New Zealand Government; 2013 [updated 2013; cited 2013]. Available from: http://www.pharmac.health.nz/ckeditor_assets/attachments/381/infosheet-08-community-pharm-budget-2013.pdf.
5. Pharmaceutical Management Agency. PHARMAC and hospital medical devices. 2013 [cited]. Available from: http://www.pharmac.health.nz/medicines/hospital-devices/.
6. Pharmaceutical Management Agency. New Zealand Pharmaceutical Schedule. 2013 [updated April, 2013; cited 2013]. Available from: http://www.pharmac.govt.nz/2013/04/03/Sched.pdf.
7. Associate Minister of Health, Minister of Health. Medicines New Zealand: Contributing to good health outcome for all New Zealanders. In: Health Mo, editor. Wellington, New Zealand: Ministry of Health, Wellington, New Zealand; 2007.
8. DeSouza R, Garret N. Access Issues for Chinese People in New Zealand Final Report. : Auckland University of Technology, Auckland; 2005 2005. Contract.
9. Babar ZUD, Pengelly K, Scahill SL, et al. Migrant health in New Zealand: Exploring issues concerning medicines access and use. Journal of Pharmaceutical Health Services Research. 2013;4(1):41-9.
10. Suin T. An holistic approach to Asian health Fourth internaitonal Asian health and well-being conference; 2010; Auckland. 2010.
11. Ragupathy R, Aaltonen K, Tordoff J, et al. A 3-dimensional view of access to licensed and subsidized medicines under single-payer systems in the US, the UK, Australia and New Zealand. PharmacoEconomics. 2012;30(11):1051-65.
12. Babar ZUD, Grover P, Stewart J, et al. Evaluating pharmacists' views, knowledge, and perception regarding generic medicines in New Zealand. Research in Social and Administrative Pharmacy. 2011;7(3):294-305.
13. Wonder M, Milne R. Access to new medicines in New Zealand compared to Australia. New Zealand Medical Journal. 2011;124(1346):12-28.
14. Milne R, Wonder M. Response to PHARMAC on access to new medicines in New Zealand compared to Australia. New Zealand Medical Journal. 2011;124(1347):18.
15. Tordoff JM, Norris PT, Reith DM. "Price management" and its impact on hospital pharmaceutical expenditure and the availability of medicines in New Zealand hospitals. Value in Health. 2008;11(7):1214-26.
16. Aaltonen K, Ragupathy R, Tordoff J, et al. The impact of pharmaceutical cost containment policies on the range of medicines available and subsidized in Finland and New Zealand. Value in Health. 2010;13(1):148-56.
17. Cumming J, Mays N, Daubé J. How New Zealand has contained expenditure on drugs. BMJ (Online). 2010;340(7758):1224-7.
18. Metcalfe S, Grocott R. Comments on "simoens, s. health economic assessment: A methodological primer. int. j. environ. res. public health 2009, 6, 2950-2966"-New Zealand in fact has no cost-effectiveness threshold. International Journal of Environmental Research and Public Health. 2010;7(4):1831-4.
19. Moodie P, Metcalfe S, Poynton M. Do pharmaceutical score cards give us the answers we seek? New Zealand Medical Journal. 2011;124(1346):69-74.
20. Moodie P, Metcalfe S, McNee W. Response from PHARMAC: Difficult choices. New Zealand Medical Journal. 2003;116(1170).
21. Grocott R. Applying Programme Budgeting Marginal Analysis in the health sector: 12 years of experience. Expert Review of Pharmacoeconomics and Outcomes Research. 2009;9(2):181-7.
22. Sage D. Report on the consultation period for the proposal to expand the functions of PHARMAC; 2010. Contract.
23. Morgan S, Boothe K. Prescription drug subsidies in Australia and New Zealand. Australian Prescriber. 2010;33(1):2-4.

24. Carlson JJ, Sullivan SD, Garrison LP, et al. Linking payment to health outcomes: A taxonomy and examination of performance-based reimbursement schemes between healthcare payers and manufacturers. Health Policy. 2010;96(3):179-90.
25. Gleeson D, Lopert R, Reid P. How the Trans Pacific Partnership Agreement could undermine PHARMAC and threaten access to affordable medicines and health equity in New Zealand. Health Policy. 2013.
26. Ragupathy R, Tordoff J, Norris P, et al. Key informants' perceptions of how pharmac operates in New Zealand. International Journal of Technology Assessment in Health Care. 2012;28(4):367-73.
27. Moynihan R. New Zealand agency comes under pressure to pay more for drugs. BMJ (Clinical research ed). 2011;342.
28. Foster RH, Wilson N. Trans-pacific partnership: Will Pharmac become a victim of its own success? BMJ (Online). 2011;343(7818).
29. Faunce TA, Townsend R. The Trans-Pacific Partnership Agreement: challenges for Australian health and medicine policies. Med J Aust. 2011;194(2):83-6.
30. Raftery JP. Paying for costly pharmaceuticals: regulation of new drugs in Australia, England and New Zealand. Med J Aust. 2008;188(1):26-8.
31. MacCormack P, Quigley J, Hansen P. Review of access to high-cost, highly specialised medicines in New Zealand. ; 2010. Contract.
32. Pharmaceutical Management Agency. Proposed pricing strategy initiatives - sole supply arrangements. Pharmaceutical Management Agency; 2002 [cited]. Available from: http://www.pharmac.govt.nz/2002/07/19/nhps.pdf.
33. Ellis CJ, Hamer AW. Cardiovascular health in New Zealand: areas of concern and targets for improvement in 2008 and beyond. New Zealand Medical Journal. 2008;121(1269):5-10.
34. Manning J. Priority-setting processes for medicines: the United Kingdom, Australia and New Zealand. J Law Med. 2011;18(3):439-52.
35. National Institute for Health and Care Excellence, . [cited]. Available from: http://www.nice.org.uk.
36. Jatrana S, Crampton P, Norris P. Ethnic differences in access to prescription medication because of cost in New Zealand. Journal of Epidemiology and Community Health. 2011;65(5):454-60.
37. Statistics New Zealand. Māori population estimates. 2012 [cited]. Available from: http://www.stats.govt.nz/browse_for_stats/population/estimates_and_projections/maori-population-estimates.aspx.
38. Ministry of Health NZG. He Korowai Oranga: Māori Health Strategy, . 2002 [cited]. Available from: http://www.health.govt.nz/publication/he-korowai-oranga-maori-health-strategy.
39. Ministry of Health NZG. The Health of Māori Adults and Children. 2013 [cited]. Available from: http://www.health.govt.nz/publication/health-maori-adults-and-children.
40. Ministry of Health NZG. Services to improve access. 2011 [cited]. Available from: http://www.health.govt.nz/our-work/primary-health-care/primary-health-care-services-and-projects/services-improve-access.
41. The United Nations WHO. The right to health. 2008 [cited]. Available from: http://www.who.int/hhr/activities/Right_to_Health_factsheet31.pdf.
42. Ministry of Business Innovation and Employment. New Zealand Government. Migration research. [cited]. Available from: http://dol.govt.nz/research/migration/monthly-migration-trends/13jun/index.asp.
43. Pharmaceutical Management Agency. Adapting the PHARMAC model for medical devices management,. 2013.
44. Pharmaceutical Management Agency. Decision criteria Pharmac; 2013 [cited 2013 11.4.13]. Available from: http://www.pharmac.health.nz/medicines/how-medicines-are-funded/decision-criteria.
45. Babar ZUD, Grover P, Butler R, et al. A qualitative evaluation of general practitioners' perceptions regarding access to medicines in New Zealand. BMJ Open. 2012;2(2).
46. Kelsey J. Hidden agendas what we need to know about the TPPA. BWB texts; 2013 [cited. Available from: www.bwb.co.nz.
47. Gallego G, Taylor SJ, Brien JaE. Funding and access to high cost medicines in public hospitals in Australia: Decision-makers' perspectives. Health Policy. 2009;92(1):27-34.
48. Pharmaceutical Management Agency. NPPA outcome report cumulative for March - December (updated 24/1/13). Pharmaceutical Management Agency,; 2013 [cited]. Available from: http://www.pharmac.health.nz/ckeditor_assets/attachments/249/summary_of_nppa_decisions_dec_12.pdf.

49. Forman J, Taruscio D, Llera VA, et al. The need for worldwide policy and action plans for rare diseases. Acta Paediatrica, International Journal of Paediatrics. 2012;101(8):805-7.
50. Lu CY, Williams K, Day R, et al.Access to high cost drugs in Australia. British Medical Journal. 2004;329(7463):415-6.
51. Lee TH, Emanuel EJ. Tier 4 drugs and the fraying of the social compact. New England Journal of Medicine. 2008;359(4):333-5.
52. Simoens S, Cassiman D, Dooms M, et al. Orphan drugs for rare diseases: Is it time to revisit their special market access status? Drugs. 2012;72(11):1437-43.
53. Munoz J, Kurzrock R. Targeted therapy in rare cancers-adopting the orphans. Nature Reviews Clinical Oncology. 2012;9(11):631-42.
54. Hudson I, Breckenridge A. The challenges of orphan drugs and orphan diseases: Real and imagined. Clinical Pharmacology and Therapeutics. 2012;92(2):151-3.
55. Drummond MF, Wilson DA, Kanavos P, et al. Assessing the economic challenges posed by orphan drugs. International Journal of Technology Assessment in Health Care. 2007;23(1):36-42.
56. Joppi R, Bertele V, Garattini S. Orphan drugs, orphan diseases. The first decade of orphan drug legislation in the EU. European Journal of Clinical Pharmacology. 2013;69(4):1009-24.
57. Largent EA, Pearson SD. Which orphans will find a home? The rule of rescue in resource allocation for rare diseases. Hastings Center Report. 2012;42(1):27-34.
58. Cheema PK, Gavura S, Migus M, et al. International variability in the reimbursement of cancer drugs by publically funded drug programs. Current Oncology. 2012;19(3):e165-e76.
59. Lu CY, Macneill P, Williams K, et al. Access to high cost medicines in Australia: Ethical perspectives. Australia and New Zealand Health Policy. 2008;5.

Identifying priority medicines policy issues for New Zealand; a general inductive study

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Abstract

Objectives: To identify priority medicines policy issues for New Zealand.

Setting: Stakeholders from a broad range of healthcare and policy institutions including primary, ~~and~~ secondary and tertiary care

Participants: Exploratory, ~~Semi~~semi-structured interviews were conducted with 20 ~~Stakeholders~~stakeholders, throughout New Zealand.

Primary and secondary outcome measures: The interviews were digitally recorded, transcribed, coded into INVIVO 10, then compared and grouped for similarity of theme. Perceptions, experiences and opinions regarding New Zealand's medicines policy issues were recorded.

Results: A large proportion of ~~Stakeholders~~ stakeholders appeared unaware of New Zealand's (NZ) medicines policy. In general, the ~~Policy~~ policy was considered to offer consistency to guide decision making. In the context of PHARMAC's fixed budget for procuring and subsidising medicines, there was reasonable satisfaction with the range of medicines available – rare disorder medicines being the clear exception. Concerns raised were~~;~~ by whom and how decisions are made and whether desired health outcomes are being measured. Other concerns included~~;~~ inconsistencies in evidence and across health technologies. Despite attempts to enable equitable access to medicines~~;~~, lower socioeconomic (including rural residents) Māori and Pacific ethnicities and, rare disorders have continued inequitable access based upon need. ~~Māori had the added issue of higher disease burden and the resultant need for an "inequity lens".~~ Other issues related to physical access, convenience to and affordability of prescribers and, the increase of prescription fees from NZ$3 to NZ$5. Concerns related to the Pharmaceutical Management Agency of New Zealand (PHARMAC) included~~;~~: a constraining budget~~,~~; non-transparency of in-house analysis~~,~~; lack of consistency in recommendations between the Pharmacology and Therapeutics Advisory Committee (PTAC) and its subcommittees~~,~~; its future ability to make autonomous decisions and affordability - with respect to both the Trans-Pacific Partnership Agreement (TPPA) and, increases in demand and cost of new medicines. Constraints and inefficiencies in the submission process to access ~~High~~ high ~~Cost~~ cost ~~Medicines~~ medicines also exist.

Conclusion: The results suggest reasonable satisfaction with the availability of subsidised medicines and ~~equitable~~ equal ability for the general population to have ~~funded~~ those medicines prescribed. However, vulnerable groups; both increasing costs of new medicines and demand; manufacturer order and evidence requirements and, some access procedures still continue to present with~~have~~ issues, not necessarily as a direct result of ~~Medicines~~ medicines ~~Policy~~ policy or PHARMAC.

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Strength and limitations of the study

Strengths

- This study is the first independent objective study to identify priority medicines policy issues, from a broad range of ~~Stakeholders~~stakeholders.
- Has identified access to medicines issues requiring further research.
- Provides a context to identified issues.
- ~~There was reasonable satisfaction with the New Zealand Medicines Policy and its principles. In particular that provision of medicines is evidence based, cost effective and there is equitable ability to have prescribed medicines listed as funded, on PHARMAC's schedule.~~
- ~~Some patient groups still experiencing difficulties in access, particularly groups with rare disorder and the low socio-economically oriented, including rural, Māori and Pacifica populations.~~
- ~~Other medicines policy issues include pharmaceutical industry's pricing of new medicines, medicines registration requirements, submission for funding process, , budgetary constraints for medicines, cultural and health literacy, patient affordability, access to prescribers and the measurement of health outcomes.~~

Limitations

- The views expressed are from 20 ~~Stakeholders~~stakeholders. Issues raised in this research project are therefore indicative. Further research is required to explore ~~the indicative~~ these issues.

Introduction

New Zealand has a population of approximately 4.5 million, with a nominal Gross Domestic Product (GDP) of approximately NZ$211 billion.(1) New Zealanders have an average life expectancy of 81.2 years, which is above the Organisation for Economic Co-operation and Development (OECD) countries average of 80.1years.(2) Just under 83 percent (82.7%) of health expenditure in New Zealand is publicly funded.(3) New Zealand's health and disability budget ~~is~~ at the commencement of this research is NZ$13.983 billion.(3) In comparison to other ~~Organisation for Economic Co-operation and Development (~~OECD~~)~~ countries, as a percentage of total expenditure on health, New Zealand spends less on pharmaceuticals.(2) Approximately NZ$795 million and NZ$280 million are available, for procuring community/cancer and hospital pharmaceuticals respectively.(4) This compares with a reported estimated spend of NZ$880 million on medical devices.(5) Approximately 1848 medicines are subsidised by PHARMAC (as listed on its Pharmaceutical Schedule)~~,~~ for use in the community~~,~~ and are largely~~mostly~~ accessible via prescription from a medical doctor.(6)

Medicines and New Zealand

Medicines make a significant contribution to health outcomes.(7) In 2007, "Medicines New Zealand" New Zealand's medicines policy, was launched in response to access concerns from the public.(7) The aim of the policy is to promote quality, effective and optimally used medicines. To guide decisions, principles of~~:~~ affordability, equity and need are stated.(7) Medicines New Zealand aims to ensure that the decisions made about prioritisation and funding are as transparent as possible, understood and open to debate. It is important for New Zealanders to have confidence that the medicines system is fair, even if they do not always agree with ~~all of~~ the decisions made (7).

Pharmaceutical Management Agency (PHARMAC)

PHARMAC, established in 1993 in response to increasing expenditure on pharmaceuticals, is a separate non-profit government agency, whose role is to determine and procure, community and oncology medicines on behalf of ~~the~~ New Zealand's 20 District Health Boards (DHBs). PHARMAC has a pre-determined fixed budget which ~~-~~it is required to operate within. In order to provide medicines considered necessary, PHARMAC employ therapeutic and economic analyses to guide decisions. ~~Their~~ PHARMAC's scope is now expanding to include hospital medicines and some medical devices.

For the majority of patients prescribed a medicine listed on PHARMAC's schedule, a NZ$5 District Health Board charge is incurred. For high user or low socioeconomic patients, access enablers~~,~~ (e.g., Prescription Subsidy, ~~such as the~~ Community Services and High User cards and, ~~now~~ recently the Services to Improve Access (SIA)) exist to help ease financial burden.(6) ~~Additional sources of government funding include: other government agencies such as (Accident Compensation Corporation (ACC)) local government, private medical cover and patient "out-of-pocket" co-payments. (6)~~

Literature exist indicating medicines issues for New Zealand related to: inequities in access, affordability, processes used and their funding. (8-31) However, no systematic work has been conducted to identify priority medicines policy issues with regards to access and funding of medicines. Within this context, it was considered timely and appropriate to conduct research that could identify priority medicines policy issues for New Zealand.

The dataset obtained from this ~~project~~ research was expected to be substantial and provide a solid platform to contribute~~ing~~ towards informing~~.~~ medicines policy, expenditure and provision and,~~ including~~ the development of optimal medicines management strategies.

Aim

The aim of this project was to identify priority medicines policy issues for New Zealand.

Methods

Study Design and Participant Selection

We conducted a general inductive study, using semi-structured exploratory interviews during December 2012-March 2013. Selection was purposeful, to ensure a broad representation of stakeholders and their opinions, who had one or more of the following traits in relation to medicines policy: involvement in its formation or implementation, had researched and/or commented on medicines policy, including having made submissions during its development, (n = 10); medically qualified doctor (n = 7, 4 of whom were active prescribers, including one of each of Māori, Indian and Asian ethnicity); medicines regulation (n = 1); representation of or, past or current involvement in medicines supply, procurement, funding or provision (excluding dispensing, n = 6); ~~belonging to one of the ethnicities in question (n = 4);~~ involved in medicines management (n = 9, one of whom was of Māori ethnicity); medical information or health technology assessment interest (n = 2); medical interest group representative (includes ethnic medical group/association, past or present, n=4); private health provision and subsidy (n = 1); patient group representative (n=2, one of whom represents and advocates for a large chronic disease group, disproportionately represented by Māori, Pacifica and increasingly the Indian and Asian ethnicity). The participants characteristics are summarised~~shown~~ in table one.

A total of 26 stakeholders were contacted and explained the research involvement. Twenty stakeholders consented and interviewed. All 20 received a "Participants Information" letter, detailing the involvement, aim and general methods. All signed a confidentiality and anonymity agreement. Fifteen interviews, were conducted face-to-face and five via telephone, due to geographical or time constraints. The average length of interview ranged from 53 – 56 minutes. No gratuity was offered.

Instrument development

The main aim of this research was to identify priority medicines policy areas. An in-depth literature review was conducted, to ascertain existing information on pharmaceutical policy. A total of 105 references were identified as useful. The following broad themes were discovered and accordingly, sets of questions developed: (1) Medicines Policy: including participant's awareness, description and opinions~~,~~; (2) Ethnicity inequities in accessing medicines (viz Māori, Pacifica and recently immigrated people whose first language was not English~~),~~); (3) PHARMAC: its pricing policy, impact upon access, economic modelling, performance, future and any improvements; (4) The Trans Pacific Partnership Agreement (TPPA): impact upon access and resultant considerations; (5) High cost medicines access; (6) Medicines policy issues not covered but considered important (see appendix one for question details)

The questions were piloted on one doctor of Māori ethnicity and one pharmacist (Ph) with an interest in medicines policy, medicines management and academia, who has previous experience in the pharmaceutical industry. Their responses were not included for analysis.

Data Collection

Participants (stakeholders) were encouraged to give comprehensive answers. Clarifying and confirming questions were asked where more information was considered necessary, or to avoid interviewer assumption.

All interviews were recorded on a voice recorder, transcribed intelligently (space fillers were omitted to enable ease of reading) Participants received their own transcript to proof, edit and approve. Only the approved editions were entered into INVIVO 10 (QSR International Pty Ltd) for coding.

Coding was conducted two ways: firstly, categorically according to answers and secondly, highlighted, grouped and compared – according to similarity of theme. Transcripts were checked for any missed issues.

A check for stakeholder bias was conducted using the coding summaries, no apparent bias was detected. Any variations appeared attributable to stakeholder knowledge~~; so were to be expected~~.

Results

Issues revealed specific to ethnicity, PHARMAC, the TPPA or high cost medicines are reported in those sections. A summary of issues is available in table two.

General Medicines Policy Issues

Nine participants stated they were unfamiliar with the policy. However, four demonstrated a tacit understanding. It was questioned how policy intentions and decisions are made, in the context of being achievable:

> *"How do you attain that?...what is the right way to make those overall policy decisions…" (pharmaceutical industry stakeholder, PI)*

All participants~~participants~~ believed medicines make a positive contribution to health. Differing levels of impact upon health were noted. There was uncertainty as to how the impact is, or could be quantified. The lost opportunity from not capturing and accessing data efficiently, was voiced by two academics for both treatment and outcomes monitoring:

> *"…we are not asking questions about patient health status before and after… so you can really see what is going on, at the GP level. Because that's at least as important as hospitalisation data". (Academic, Ac)*

Conversely, one participant said he would prefer to see more investment in epidemiology, as opposed to increasing the medicines budget, in a desire to preserve health.

Low socioeconomic patients were considered to have a higher burden of disease. Affordability to prescribers was described as the major issue, which may be compounded by the 2013 raise in prescription co-payment from NZ$3 to NZ$5. ~~Australia was contrasted, where there are comparatively low preseriber and higher prescription co-payments.~~

Despite access enablers, such as the High User Cards and Community Services Cards, it was questioned whether those in need are utilising them. One General Practitioner (GP) said cost-sensitive patients could be managed with prudent prescribing and education on priorities:

> *"You could get all your medicines for less than a pack of cigarettes. It's educational priorities and various other things, where the effort needs to go rather than reducing the cost much further." (GP)*

The opposite situation of the misuse of access ~~enablers~~ was described:

> ~~*"So it's that whole inverse law." (GP);*~~
>
> *"I initially struggled to understand how somebody could pull up outside a pharmacy in a Mercedes Benz and… present their scripts for their family and handover their Community Services Card… As soon as they get in the country; they put the money into a family trust...So the wealth of the individual gets assessed, which qualifies them for a Community Services Card and then they wave that around." (Pharmacist, Ph)*

Sole supply[1] provision raised issues in terms of: supply outages when switching supplier (and having to pay for the alternative option)~~,~~ options for patient intolerance and vulnerability if a significant disruption in supply occurs (e.g., a disaster destroying a supplier's warehouse).

There was additional reference to policies and funding needing to be consistent and interlink, especially for priority areas. "Quit-Line" was given as an example: a $40 million funded smoking cessation programme, described as having markedly less evidence than the appropriate medicines (which were ~~as~~ not funded for many years). Budgetary constraints were the reason given for this. Equally, the government funding of "alternative medicines" was described as something which needed debate~~needing debate~~:

> "*… government is providing funding for people to obtain alternative medicines … real debate to be had… money better spent some place else in the healthcare system?*" *(Pharmceutical Industry, PI)*

One Doctor voiced frustration at PHARMAC's Therapeutic Advisory Committee (PTAC) [2] being *"generalists"* who over-ride the recommendation of their subcommittee. With patient sub-typing and genomic medicine on the horizon, he considered *"generalists"* may not understand what they are assessing and dismiss research, thereby inhibiting access. Access is then through an ability to pay for litigation and decided by a non-medical expert.

With demographic changes increasing demand for healthcare services and a general movement towards increasing costs of new medicines, there was concern for future affordability of medicines. It was ~~S~~suggested~~ion was~~ funding may move away from being population based~~,~~and move toward funding health outcomes. ~~The biggest concern being: was the discovery and affordability of a panacea.~~ An academic suggested~~:~~ changes in co-payments, taxation or medicines classification status may result. The oncologist had concerns that the lack of research (research being an attracter) being conducted within New Zealand, will compound the low availability of future medical specialist prescribers. Extending prescribing ability to non-doctors was considered to help. However, for oncology, a medical specialist was still considered to ~~still~~ be required to make treatment decisions.

Ethnicity Issues

Most issues presented, related to socio-economic variables and are presented under General Medicines Policy Issues.

[1] Sole supply arrangements are likely to be used by PHARMAC in markets where generic competition exists, resulting in there being only one brand of a particular chemical listed. It is possible that PHARMAC would agree preferred supplier status for some chemicals in exchange for price concessions, affecting access to related pharmaceuticals within the same therapeutic group.~~(32.~~ Pharmaceutical Management Agency. Proposed pricing strategy initiatives - sole supply arrangements. Pharmaceutical Management Agency; 2002 [cited]. Available from: http://www.pharmac.govt.nz/2002/07/19/nhps.pdf.

[2] PTAC is PHARMAC's primary clinical advisory committee. PTAC's role is to provide clinical advice to the Board of PHARMAC.

Those with poor English speaking skills were described as having access to an English speaking relative or even interpreters if needed:

> *"I think if they can access General Practice or the Hospital system, their access to the medications is just as good as anybody else's. I'm not aware of any specific ethnic problems in accessing our medicines.." (GP)*

One GP felt strongly that Māori and Pacifica access inequities are ~~evidenced by~~evident and are reuslting in poorer health outcomes. He considers his colleagues are treating everyone the same but with inequitable risk: earlier intervention, improved communication, education and patient engagement are required~~:~~

> *"I think the key issue is the prescribers have a poor understanding of inequalities. Because, the prescribers generally approach things as; I treat everyone the same… they must have an inequity lens on anyone they see… but if the quality of your discussion and the quality in the way in which you prescribed that was poor i.e., you culturally are incompetent and you have a disconnect with the patient…" (GP)*

Other issues related to Asian ethnicities wanting treatment (oncology setting) irrespective of likely outcomes and~~,~~ the use of alternative treatments e.g., St John's Wort or Vitamin C injections impacting upon medical treatment. One of the doctors had issue with alternative practitioners recommending such treatments~~, as safe and evidence based, upon requesting information to support these treatments, he found the paper to be an out-dated and flawed case study.~~

Pharmaceutical Management Agency (PHARMAC)

There was general appreciation shown towards PHARMAC's strategy of creating competition in order to achieve a lower purchasing price. This was seen as advantageous for the purchasing of a greater range of medicines, in the context of a fixed budget.

The budget was defined as the threshold for provision, which was considered too small by an academic and pharmaceutical industry stakeholder ~~PI~~, causing a focus on cost as the driver of value and provision, thereby contributing toward *"static efficiency"*:

> *"If Pharmac's objective is to stay within budget then it's doing well… improve the health of New Zealanders within a capped pharmaceuticals budget…it's doing moderately well…objective were to improve the health of New Zealanders taking into account the financial constraints of Vote Health…it's doing poorly because it should be fighting for a better share of Vote Health." (Ac)*

A public service~~ant~~ stakeholder (PS) offered the ~~made the~~ following standpoint~~comment~~: *"You can always achieve more with more."* ~~(~~I~~i~~n terms of a bigger budget) but ~~there isn't~~ an analytical framework is not in place that~~which~~ would define whether the medicines budget receives a fair proportion of "Vote Health~~.~~" or not.

~~There was concern: whilst~~ PHARMAC's budget is determined at regular defined intervals~~,~~ but medicines enter the marketplace sporadically~~, for which funds may not be available~~. This caused concern for the oncologist, that extra funds may not be available to deal with this.

The private health care organisation stakeholder~~provider~~ (PHCO~~P~~) thought PHARMAC's approach of requiring new and more expensive medicines to be better than standard medicines a~~:~~ *"completely acceptable*

approach." It was suggested by the PHCOP and an academic that their approach could be more widely adopted, both overseas and with the expansion of PHARMAC's role to medical devices:

"…the expertise PHARMAC has built up…is something that we could learn from and borrow from, for the wider health sector… I'd like to see them take on medical devices, because that is absolutely scandalous that these products are getting onto the market without being properly evaluated…" (Ac)

There was caution given from one pharmacist that PHARMAC's expansion into hospital medicines (in an acute care setting of moribund disease) may limit choices. Concern was shown for risk, if New Zealand is world-leading in this type of provision.

Provision was described as having a utilitarian focus: *"The greatest good for the greatest number" (PS)* and described as being: you get what you need - not what you want. One doctor questioned whether the lost opportunity from not treating someone is being measured. Rare disorder patients were mentioned and are discussed under High Cost Medicines. The distinction was made that provision of a medicine in a cost effective manner, which PHARMAC achieves, is not the same as delivering healthcare:

> *"I find some of their PR a little bit irritating…bray on about the marvellous healthcare they're delivering… delivering medicines in a cost effective manner but that's not saying it's delivering healthcare…" (PHCOP)*

~~The q~~Questioning ~~of the~~ PHARMAC's undertaking of economic modelling ~~. Pharmac undertakes~~ received very favourable comments from 15 of the participants. Three participants were not familiar with economic modelling:

> *"I think it's world leading actually. No one else dares do it. That's the crazy thing. Here we are little old New Zealand and we dare do it." (Ac);*
>
> *"Well I mean, as a tax payer you could argue that for the majority of the products they get in, they've done a really good job of driving cost out of the system." (PI);*
>
> *"Technically it's very good. PHARMAC considers clinical effectiveness and cost effectiveness.. they make trade-offs… they look at the QALYs and the number of people affected and how their quality of life will be improved and so on, I think is a very good model." (PS)*

It was suggested that the cost-benefit ~~should be a consideration because~~ of ~~valuing the~~ returning ~~of~~ an individual back to their normal daily activities should be considered by PHARMAC, such as what Accident Compensation Corporation (ACC) does ~~i~~when assessing intervention options.

A pharmaceutical industry ~~representative~~ stakeholder and academic were concerned the required economic modelling submitted by suppliers is adjusted with unknown *"in-house"* variables, making it hard for suppliers to understand decisions. This was contrasted against Medsafe's practice, where decision modelling is transparent:

> *"Pharmac receives a dossier from the company…Assumptions of statistical models get changed…QALYs get changed…population who will use the product get changed…that should be part of a scientific debate…companies don't know what information is being used to make the decisions on their products…we would like a right of reply to those… It happens with MedSafe…Not as though it could potentially negatively affect evidence based decisions."(PI)*

Delays in the submission process of up to eight years and described as a~~:~~ *"medicines waiting list,"* were of concern for an academic, pharmaceutical industry stakeholder~~representative~~ and patient group representative (PGR), who all thought access was related to cost. There was suggestion from one academic to follow Australia's submission process and out-source assessments from independent bodies.

Trans Pacific Partnership Agreement (TPPA)

Very few participants were familiar with the TPPA, some referred to speculation and no facts. One participant refused to give any comments related to the TPPA.

There was acknowledgement that trade deals are complex and often require compromises and trade-offs. New Zealand was referred to as a~~:~~ *"small country"* and *"we need our trade partners."* There was concern that already "*big amounts"* are being spent on healthcare and the *"benefits are low"* and, if there is a resultant increase in the cost of medicines, where would resources come from, to off-set any cost increases.

The main issues were: (1) Patent extension, delaying generic entry to market, thereby prolonging a higher cost of provision; (2) Industry influencing supply (described as an issue of sovereignty) may result in quicker access to new medicines but also an increase in public campaigns and appeals processes if PHARMAC's decisions are unpopular with the pharmaceutical industry or patient groups; and, (3) T~~he call for t~~ransparency requests in PHARMAC's assessment process caused the most concern and confusion. One ~~Academic~~ academic said he didn't think PHARMAC could be more transparent and that transparency might mean the industry discloses its pricing processes and the results of all clinical trials.

In general, scepticism was voiced as to what the driving force is behind the agreement and what the benefits would be for New Zealand - with the United States of America (USA) being a protected market (heavily subsidised). Australia was described by a pharmaceutical industry representative~~,~~ as getting *"trounced"* over their agreement with the USA, losing a lot of their pharmaceutical production and jobs as a result:

> *"Forget it…wouldn't even bother going along to the negotiations"(~~PHCP~~PHCO);*
>
> *"…tell the US to bugger off quite frankly. You either put everything on the table and we talk about it or no, you don't…We should learn from what happened in Australia…" (PI)*

Conversely, another participant suggested whilst *"America"* has influence, it may become limited as a result of the influence of China's developing economy and differing ideas around protection and, new opportunities may develop:

> *"…a hugely developing economy in the form of China that basically has total disregard for such things… so the ability for America…is probably going to be limited in the world of the future, and maybe different forms of protection of ideas will kind of evolve… it's very hard to predict how the market might respond or what kind of new opportunities develop." (PS)*

~~One academic suggested that PHARMAC's monopsony is an anathema to the USA.~~ A pharmaceutical industry stakeholder~~representative~~ said Medicines New Zealand (New Zealand's prescription medicines representative association, same title as the policy) is attempting to ensure its United States of America~~USA~~ equivalent understands New Zealand's medicines system:

> *"…working quite hard to ensure…our sister organisation in the US is effectively asking the US government to achieve out of the process, is well enough informed to understand actually what the New Zealand model does achieve, what it doesn't achieve and how that can be improved…So we're working hard to make sure it's a process that actually benefits New Zealanders as well, and all of the transparency, timeliness, appeals – those aspects that we've discussed, are exactly I think what the US is likely to be asking for." (PI)*

Most considered that New Zealand's current ability to access generic medicines or, independence in procuring medicines should be upheld. If not, funds may need to be redirected from other services or, patient co-payments would need to rise, in order to compensate a likely increase in the cost of medicines.

High Cost Medicines

A GP questioned the necessity of continuing the Special Authority (SA)[3] status for a medicine, once the appropriate use of a medicine has been established. Not all participants were familiar with the Named Patient Pharmaceutical Assessment (NPPA)[4] access scheme. Most high cost medicines were described as being *"breakthrough"* or *"expensive"* and are restricted, to control spending. One ~~participant~~ participant said, if it was *"dirt cheap,"* there would be "*no argument,"* indicating the case even if the medicine didn't have clear health benefits:

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> *"My bet, is that PHARMAC would listen to anyone that agrees with them saying no. Because it's expensive ….They are diametrically opposed for a reason and the reason is cost." (Ph);*
>
> *"The Rabbits in charge of the lettuce patch" (M)*

One public ~~servant~~ service stakeholder thought there to be no inappropriate blocking of access to medicines, as no complaints about access have been received at their level. Equally, another ~~Public~~ public ~~Servant~~ service stakeholder commented that there are patients accessing medication costing up to NZ$500, 000 per year:

> *"…So it's not that the system can't cope with treatments that are high cost, it's just that we would expect a return for that cost and for it to be justifiable in terms of what we value." (PS)*

A small group of patients were described as not having access to high cost medicines. Access was described as *"the collective good."* Conversely, *"people dying from a lack of access to very cheap and simple therapies"* ~~were~~ was described. It was suggested that it is a DHB's remit to look after its population, highlighting the issue of population versus individual access. An assertion ~~statement~~ was made~~.~~, if are we advocating treatment at any cost ~~and if so~~, who pays:

> *"It's a question of who pays for all these things. I think if you have pretence; like there is in the USA, that cost isn't of any relevance… then you're going down the wrong path." (PHCP)*

The oncologist described the NPPA process as inefficient~~.~~ ~~:~~ a comprehensive and referenced application, takes him up to six~~6~~ hours, potentially impacting on his clinic time and perversely hindering patient access.

[3] Special Authority criteria define the clinical circumstances of patients who can receive funding for the medicine. People may first be required to try a less expensive medicine or the medicine may need to be prescribed by a particular type of health practitioner.

[4] NPPA is a mechanism to give individual named patients access to medicines they need, but which aren't funded on the Pharmaceutical Schedule. NPPA replaces the three Exceptional Circumstances (EC) schemes that PHARMAC previously managed.

He suggested PHARMAC at a nominal cost could employ someone to aid in information gathering and in the process develop expertise.

Additionally, the oncologist believes oncology has the stigma that everyone dies but individual survival may be greater than the median survival assessment. This issue was presented in comparing the availability of two to three ~~2-3~~ drugs in Australia that are~~–~~ unavailable in New Zealand.

Questions were posed: (1) Is it fair to give fourth 4th or fifth~~5~~th line chemotherapy and not give a first line treatment e.g. for rare disorders? and, (2) When do you stop treatment, a patient was described as gaining access to expensive medication, their condition was fragile and they died a few weeks later:

> *"I think it's important if Pharmac has a few loose strings in terms of hospital and severe rare conditions. They are perhaps because of how they are funded, they want a very narrow perspective on those, to try and avoid blow out. They are very emotive issues we don't always know how to best manage people's care. " (Ph)*

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High need patients, such as rare disorder patients received the most sympathy for difficulty in access because of the exclusion criteria. Evidence requirements were described as difficult to attain due to low patient numbers. Conversely, the ~~PHCP~~ PHCO suggested the supplier needs to produce quality evidence:

> *"…where there's some evidence, but not solid or quality evidence: … the company doing the – providing the medication, it behoves on them to do some research in those areas and produce quality data."(PHCO~~P~~)*

Discussion

We purposefully attempted to be open to issues and their capture, despite some issues already being identified. Our focus was on access to medicines. It is possible there are other issues in existence, we neither recognised, nor captured. We did not seek to determine issues specifically related to generic medicines, considered a "*vital component of New Zealand's medicine cost management policies*" by Babar et al.(12)

Medicines Policy

Medicines are clearly valued health interventions: evidenced by the budget, literature and responses from stakeholders. The smaller percentage spend on pharmaceuticals in New Zealand (described as a constrained budget) compared with similar countries such as Australia, the United Kingdom (UK) and the USA,(2) may in fact reflect the price reduction strategies that are implemented by PHARMAC(32), as opposed to less opportunity to improve health outcomes. However, this needs to be tested through robust research on health outcomes and their relationship to pharmaceutical spending.

Delayed access and the resultant impact discussed by some of the participants, was also described by Ellis and Hamer,(33) in relation to New Zealand's statin availability for atherosclerotic patients, as probably negatively impacting health outcome and considered to be due to the capped budget. They considered this *"anomalous,"* as other types of health care are not capped. This anomaly was also described by a number of participants but may change with PHARMAC's expanding role.

New medicines are increasing in costs along with demand, causing tension in affordability. Price efficiency initiatives, such as what PHARMAC encourages, help ease the tension in affordability of provision. Another option is to reduce demand, ~~:~~ either through "gate keeping" (not usually a popular choice) or genuine effects,

such as initiatives to maintain health or prevent disease. We assume a reduction in demand and therefore burden of provision, should result in healthcare becoming more affordable, for providers and helping those remaining in need.

~~.~~Manning,(34) compared the processes of~~:~~ decisions, pricing, economic analysis, provision and access and, participation and appeals between the UK, Australia and New Zealand. It was suggested that resolving issues may benefit from a disputes panel comprising a broad range of experts in~~:~~ scientific, economic, policy and ethical evaluations, in order to provide an objective decision. Manning additionally reported that approximately one sixth of the United Kingdom's ~~United Kingdom's~~ National Institute for Healthcare and Clinical Excellence (NICE) recommendations are appealed and upheld.~~.~~(35) There shouldn't be great demand, if evaluation processes are robust.

Ethnicity

Our participants revealed that low socioeconomically related populations (encompassing Māori and Pacifica people) are continuing to have access issues related to financial, structural, educational and cultural barriers. These findings were consistent with that of Jatrana et al,(36) who assessed SOFIE-health's 18320 respondents (an add-on to Statistics New Zealand's longitudinal survey of Family, Income and Employment). Māori and Pacific people were more likely to defer purchasing a prescription due to cost, which at that time was NZ$15.

Māori represent approximately 15 percent of New Zealand's population(37) and on average have the poorest health status of any ethnic group in New Zealand.(38, 39) Pacifica people represent 6.5 percent of the population and also experience health inequalities. He Korowai Oranga: the Māori Health Strategy (2002),(38) recognises the Treaty of Waitangi principles of: partnership, participation and protection, through which, the aim is to reduce existing health inequalities. This aim is extended to include the Pacific people, who like Māori, they are over represented by a low socioeconomic situation, reflecting low affordability and health literacy, which in turn affects access. The Ministry of Health recently launched Services to Improve Access(SIA)~~,~~(40) an additional targeted capitation payment, available to p~~P~~rimary h~~H~~ealthcare o~~O~~rganisations(PHOs) to reduce health inequalities. It is designed for new services (e.g., outreach programmes) or improving access (e.g., funding transport) for Māori, Pacific people and those of low socioeconomic status. Once SIA is embedded, it would be prudent to evaluate its impact.

Other Ministry of Health initiatives, such as Whanau Ora (to build the health, participation and capability of families) and~~,~~ One Heart Many Lives (to improve the cardiac health of Māori and Pacific men) along with recent changes in health practitioner training, appear good initiatives for engaging Māori and Pacifica in a culturally appropriate way. It would be prudent to evaluate their impact.

We did not have any issues specifically described for new immigrants. It was described to us that patients with poor English speaking capability, present to practitioners with an English speaking person, or frequent a surgery of their ethnicity. This is at odds with Babar et al,(9) who found for 11 Chinese and Indian migrants, residing in New Zealand for less than five years, financial barriers existed in affording doctors, pharmacists and medicines and, that language barriers exist. This anomaly may highlight the differences in both the perspective and experience of the stakeholders we interviewed.

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Asia and India have different medicines access systems to New Zealand. Babar et al additionally found there is a lack of information on New Zealand's medicines system, including medicines provision and classifications. The United Nations and World Health Organisations, when discussing the right to health,(41) refer to migrants as being vulnerable to reduced access to health services for reasons that include: language or cultural barriers. New Zealand has a significant migrant population, reported as 927 000 in 2006.(42) The current main countries for immigration are: China (15%), United Kingdom (unspecified), India (13%) and the Philippines (8%).(42) In consideration of Babar et al's work and immigration statistics, there may be a significant number of people from these countries with issues, resulting in difficulties in accessing healthcare and therefore medicines.(8-10) In light of this, it may be worth further investigating new immigrant issues.

PHARMAC

The general appreciation for New Zealand's needing to be efficient, in order to provide more medicines, expressed by our stakeholders, was also shown by Ragupathy et al.(26) Included, was the need to apply consistent economic evaluations to other health technologies, to support congruous decisions for resource allocation. PHARMAC's expansion into procuring hospital medicines and medical devices may enable greater consistency of evaluation across technologies.

The significance of PHARMAC's role expansion should not be underestimated. PHARMAC will need to practice caution with expanding their role into hospitals, which are generally settings of acute and moribund disease. We are unaware whether a closed formulary has occurred elsewhere in the world. PHARMAC have been noted to have consulted directly with medical specialist groups to discuss their role expansion, including consultation on hospital medical devices,(43) so would appear to be fully cognisant and appreciative of this issue.

We found delays of up to eight years, in PHARMAC's process for funding medicines onto the Pharmaceutical Schedule, which a number of our participantsstakeholders purported to be due to the medicine's pricing and/or PHARMAC's budget not being able to expand. Other reasons may be the medicine's priority status, insufficient information or, not meeting PHARMAC's nine decision criteria.(44) The question is whether this means delays in therapeutic advancement and therefore improved health outcomes.

The measurement of opportunity foregone was of clear concern to the Oncologist oncologist we interviewed. New Zealand has a capped medicines budget, it cannot expand and therefore drives the need for efficient spending (determined using cost utility analysis, where medicines are assessed against QALY gains per NZ$1 million) Using this process for provision means there is opportunity foregone, as described by Milne and Wonder.(14) We are not aware of New Zealand focused research assessing either opportunities foregone or, other specialist viewpoints on access. The exceptions being: Ellis and Hamer(33) in 2008, discussing the delayed availability of cardiac medicines; MacCormack et al in 2009,(31) assessing stakeholders views on needed access to high cost medicines and; The Sage report for the Ministry of Health in 2010, (22) reporting the consultation of stakeholders on the proposal to expand PHARMAC's role.

Sole supply issues (supply outages, lack of palatable formulations, resultant out of pocket payments for alternatives and vulnerability as a result of a disaster) were reported as still continuing, despite there being penalties for suppliers. This was also reported by Babar et al,(45) who reported additional concerns with poor quality products in the past, from previous studies.

Trans-Pacific Partnership Agreement (TPPA)

There is very little information available on the TPPA, the reason given: particulars of the negotiation are changing. What does exist concurs with our findings, ~~:~~ it questions the motivation and self-interest of parties involved and warns of possibly binding impacts that may affect health services budgets and PHARMAC's autonomy, including method of procurement and provision.(25-29, 46) Such impacts stem from the USA's desire for stricter protection of intellectual property rights, transparency of in-house evaluation, regulatory coherence, dispute settlement, government procurement and evidence based decisions being contestable in court. Unless budgets ~~are~~ expand~~ed~~ to cope with likely increases in costs, there may need to be a re-evaluation of provision, subsidies and co-payments. In contrast to existing publications, our research additionally suggested a TPPA may enable earlier access to newer medicines. It may be of use to quantify what effect ~~-~~a TPPA would have upon medicines access.

High Cost Medicines

A significant issue discussed in our study, was the need to differentiate between high cost medicines and both highly specialised needs ~~or~~ and medicines in relation to the NPPA access scheme. McCormack et al, (31) suggest a medicine that costs NZ$20 000 per patient per year may be considered high cost. It is important to be cognisant of the total cost to the health system of any medicine, which is dependent upon the number of patients treated (volume used) and the acquisition price. Some high cost medicines may not result in a high total cost to PHARMAC, for some patient groups. Gallego et al,(47) question how treating large populations at high total cost for small population gains, compares with treating smaller populations, for possibly significant benefit.

The issue of treating large versus small populations may intensify with patient subtyping and genomic medicines development (as described by the oncologist) where greater expectation to fund (i.e., demand) may occur. With the NPPA process now reported in our findings, as enabling the capturing of cancer patient subtype information, cancer medicines outcomes may become easier to measure and if positive, make it harder to decline funding treatments. It may also mean the table is turned and large populations end up having limited treatment options, if outcomes cannot be measured in the same way. However, funding outcomes will give a clear indication for innovation and direction to both suppliers and funders of medicines.

Our findings describe both the SA and the NPPA access schemes as being inefficient. The SA inefficiency finding is also supported by Babar et al's evaluation of GP perceptions on access to medicines in New Zealand.(45) Once correct prescribing of a medicine has been established it may not be necessary to continue a medicine's SA status. The NPPA process appears to impact significantly on consultant clinic time which may perversely hinder patient access. With demographic trends indicating greater demand for such medicines, the impact of the inefficiency may intensify. PHARMAC's website lists 555 approvals and 15 declines for NPPA access.(48) The high rate of NPPA approvals, brings into question the need for such a process. Alternatively, i~~I~~t may be more efficient for PHARMAC, at a nominal cost, to contract an evidence based facilitator, to ease the burden of application for clinicians.

Difficulty in access to high cost medicines, encompassing rare disorder patients, as described by our participants, has been widely documented.(31, 47, 49-58) The nature of rare diseases makes it hard to gain the necessary evidence PHARMAC requires for evaluation. This issue is compounded for suppliers because the need to satisfy both manufacturer ordering and regulatory requirements, adds to the unit cost of supply for low volume demand medicines. It may be worth investigating options to reduce cost of supply and

provision in the context of constrained evidence. PHARMAC have recently sought public and professional input into its decision criteria. The results have yet to be published but may reveal new options or initiatives.

Our research highlighted the issue of access to medicines of therapeutic value in the context of a fixed predetermined budget and the difficulties in how priorities for funding are determined. Lu et al,(59) in discussing ethical perspectives to the access of high cost medicines in Australia, discussed the issue of having equal need requires equal opportunity to access care and suggest where evidence requirements are not achieved, treatment commence on a trial and outcome basis. This does come with ethical concerns but may enable both access and capturing evidence. MacCormack et al,(31) suggest "risk sharing" supply to ensure some form of access (defining a threshold for maximum numbers to treat for a high cost medicine, above which, the supplier funds)

Conversely, Simoens et al,(52) caution providing access to medicines with limited effectiveness, implies rare disorders health improvement is more valuable than a common disease, which challenges the utilitarian view of: the health gain of each patient is valued equally. With both increasing effort in the development and availability of orphan drugs, this issue may only worsen. Equally, other questions arise: because we see the ill health, can't mean preferential treatment over someone who has a "silent" state of declining health. There are people not getting access to inexpensive medicines, who are at risk, as stated by an academic. Perhaps remedying issues of access based on need, could start with prioritising based on the impact of an unmet need?

Conclusion

Overall, despite issues being identified, there was reasonable satisfaction with the New Zealand's ~~Medicines~~ medicines ~~Policy~~ policy and its principles. In particular that provision is evidence based, cost effective and there is equitable ability to have prescribed medicines listed as ~~funded~~subsidised, on PHARMAC's schedule.

However, despite this, there appears to be some patient groups still experiencing difficulties in access, not necessarily appearing as a result of medicines policy or PHARMAC. Such groups being~~:~~ rare disorders and the low socio economic (encompassing rural, Māori and Pacifica populations). Other issues ranged from: the pharmaceutical industry's pricing of new medicines~~,~~ ; manufacturer and registration requirements~~,~~ ; the submission for funding process~~,~~ ; increasing demand for medicines and the resultant financial impact~~costs,~~ ; budgetary constraints~~,~~ ; cultural and health literacy~~,~~ ; patient affordability and access to prescribers~~,~~ ; through to knowledge development for clinical expertise and the measurement of health outcomes.

Our study has highlighted issues in access based upon need and the consequences of unmet need. The context being~~:~~ a fixed~~,~~ and predetermined budget~~, and~~ increasing demand~~,~~ and the rising cost of medicines ~~is causing~~are all compounding constraints in affordability. We suggest these issues and consequences of unmet needs may worsen and options for demand and provision may need to be explored further.

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Authors contribution: ZB conceptualised and designed the study. The data collection, entry and analysis was handled by SF and ZB. SF and ZB wrote the manuscript. The final version is approved by all authors. ZB acts as an overall guarantor to this study.

Transparency: ZB affirms that the manuscript is an honest, accurate, and transparent account of the study being reported; that no important aspects of the study have been omitted.

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References

1. Statistics New Zealand. Top Statistics. 2013 [cited]. Available from: http://www.stats.govt.nz/browse_for_stats/snapshots-of-nz/top-statistics.aspx.
2. Organisation for Economic Co-operation and Development countries. OECD Health Data 2013 - frequently requested data: health expenditure. 2013 [cited]. Available from: http://www.oecd.org/els/health-systems/oecdhealthdata2013-frequentlyrequesteddata.htm.
3. Ministry of Health. Funding. Ministry of Health; 2013 [cited 2013]. Available from: http://www.health.govt.nz/new-zealand-health-system/funding.
4. Pharmaceutical Management Agency. Setting and managaing the combined pharmaceutical budget (CPB). New Zealand Government; 2013 [updated 2013; cited 2013]. Available from: http://www.pharmac.health.nz/ckeditor_assets/attachments/381/infosheet-08-community-pharm-budget-2013.pdf.
5. Pharmaceutical Management Agency. PHARMAC and hospital medical devices. 2013 [cited]. Available from: http://www.pharmac.health.nz/medicines/hospital-devices/.
6. Pharmaceutical Management Agency. New Zealand Pharmaceutical Schedule. 2013 [updated April, 2013; cited 2013]. Available from: http://www.pharmac.govt.nz/2013/04/03/Sched.pdf.
7. Associate Minister of Health, Minister of Health. Medicines New Zealand: Contributing to good health outcome for all New Zealanders. In: Health Mo, editor. Wellington, New Zealand: Ministry of Health, Wellington, New Zealand; 2007.
8. DeSouza R, Garret N. Access Issues for Chinese People in New Zealand Final Report. : Auckland University of Technology, Auckland; 2005 2005. Contract.
9. Babar ZUD, Pengelly K, Scahill SL, et al. Migrant health in New Zealand: Exploring issues concerning medicines access and use. Journal of Pharmaceutical Health Services Research. 2013;4(1):41-9.
10. Suin T. An holistic approach to Asian health Fourth internaitonal Asian health and well-being conference; 2010; Auckland. 2010.
11. Ragupathy R, Aaltonen K, Tordoff J, et al. A 3-dimensional view of access to licensed and subsidized medicines under single-payer systems in the US, the UK, Australia and New Zealand. PharmacoEconomics. 2012;30(11):1051-65.
12. Babar ZUD, Grover P, Stewart J, et al. Evaluating pharmacists' views, knowledge, and perception regarding generic medicines in New Zealand. Research in Social and Administrative Pharmacy. 2011;7(3):294-305.
13. Wonder M, Milne R. Access to new medicines in New Zealand compared to Australia. New Zealand Medical Journal. 2011;124(1346):12-28.
14. Milne R, Wonder M. Response to PHARMAC on access to new medicines in New Zealand compared to Australia. New Zealand Medical Journal. 2011;124(1347):18.
15. Tordoff JM, Norris PT, Reith DM. "Price management" and its impact on hospital pharmaceutical expenditure and the availability of medicines in New Zealand hospitals. Value in Health. 2008;11(7):1214-26.
16. Aaltonen K, Ragupathy R, Tordoff J, et al. The impact of pharmaceutical cost containment policies on the range of medicines available and subsidized in Finland and New Zealand. Value in Health. 2010;13(1):148-56.
17. Cumming J, Mays N, Daubé J. How New Zealand has contained expenditure on drugs. BMJ (Online). 2010;340(7758):1224-7.
18. Metcalfe S, Grocott R. Comments on "simoens, s. health economic assessment: A methodological primer. int. j. environ. res. public health 2009, 6, 2950-2966"-New Zealand in fact has no cost-effectiveness threshold. International Journal of Environmental Research and Public Health. 2010;7(4):1831-4.
19. Moodie P, Metcalfe S, Poynton M. Do pharmaceutical score cards give us the answers we seek? New Zealand Medical Journal. 2011;124(1346):69-74.
20. Moodie P, Metcalfe S, McNee W. Response from PHARMAC: Difficult choices. New Zealand Medical Journal. 2003;116(1170).
21. Grocott R. Applying Programme Budgeting Marginal Analysis in the health sector: 12 years of experience. Expert Review of Pharmacoeconomics and Outcomes Research. 2009;9(2):181-7.
22. Sage D. Report on the consultation period for the proposal to expand the functions of PHARMAC; 2010. Contract.
23. Morgan S, Boothe K. Prescription drug subsidies in Australia and New Zealand. Australian Prescriber. 2010;33(1):2-4.

24. Carlson JJ, Sullivan SD, Garrison LP, et al. Linking payment to health outcomes: A taxonomy and examination of performance-based reimbursement schemes between healthcare payers and manufacturers. Health Policy. 2010;96(3):179-90.
25. Gleeson D, Lopert R, Reid P. How the Trans Pacific Partnership Agreement could undermine PHARMAC and threaten access to affordable medicines and health equity in New Zealand. Health Policy. 2013.
26. Ragupathy R, Tordoff J, Norris P, et al. Key informants' perceptions of how pharmac operates in New Zealand. International Journal of Technology Assessment in Health Care. 2012;28(4):367-73.
27. Moynihan R. New Zealand agency comes under pressure to pay more for drugs. BMJ (Clinical research ed). 2011;342.
28. Foster RH, Wilson N. Trans-pacific partnership: Will Pharmac become a victim of its own success? BMJ (Online). 2011;343(7818).
29. Faunce TA, Townsend R. The Trans-Pacific Partnership Agreement: challenges for Australian health and medicine policies. Med J Aust. 2011;194(2):83-6.
30. Raftery JP. Paying for costly pharmaceuticals: regulation of new drugs in Australia, England and New Zealand. Med J Aust. 2008;188(1):26-8.
31. MacCormack P, Quigley J, Hansen P. Review of access to high-cost, highly specialised medicines in New Zealand. ; 2010. Contract.
32. Pharmaceutical Management Agency. Proposed pricing strategy initiatives - sole supply arrangements. Pharmaceutical Management Agency; 2002 [cited]. Available from: http://www.pharmac.govt.nz/2002/07/19/nhps.pdf.
33. Ellis CJ, Hamer AW. Cardiovascular health in New Zealand: areas of concern and targets for improvement in 2008 and beyond. New Zealand Medical Journal. 2008;121(1269):5-10.
34. Manning J. Priority-setting processes for medicines: the United Kingdom, Australia and New Zealand. J Law Med. 2011;18(3):439-52.
35. National Institute for Health and Care Excellence, . [cited]. Available from: http://www.nice.org.uk.
36. Jatrana S, Crampton P, Norris P. Ethnic differences in access to prescription medication because of cost in New Zealand. Journal of Epidemiology and Community Health. 2011;65(5):454-60.
37. Statistics New Zealand. Māori population estimates. 2012 [cited]. Available from: http://www.stats.govt.nz/browse_for_stats/population/estimates_and_projections/maori-population-estimates.aspx.
38. Ministry of Health NZG. He Korowai Oranga: Māori Health Strategy, . 2002 [cited]. Available from: http://www.health.govt.nz/publication/he-korowai-oranga-maori-health-strategy.
39. Ministry of Health NZG. The Health of Māori Adults and Children. 2013 [cited]. Available from: http://www.health.govt.nz/publication/health-maori-adults-and-children.
40. Ministry of Health NZG. Services to improve access. 2011 [cited]. Available from: http://www.health.govt.nz/our-work/primary-health-care/primary-health-care-services-and-projects/services-improve-access.
41. The United Nations WHO. The right to health. 2008 [cited]. Available from: http://www.who.int/hhr/activities/Right_to_Health_factsheet31.pdf.
42. Ministry of Business Innovation and Employment. New Zealand Government. Migration research. [cited]. Available from: http://dol.govt.nz/research/migration/monthly-migration-trends/13jun/index.asp.
43. Pharmaceutical Management Agency. Adapting the PHARMAC model for medical devices management,. 2013.
44. Pharmaceutical Management Agency. Decision criteria Pharmac; 2013 [cited 2013 11.4.13]. Available from: http://www.pharmac.health.nz/medicines/how-medicines-are-funded/decision-criteria.
45. Babar ZUD, Grover P, Butler R, et al. A qualitative evaluation of general practitioners' perceptions regarding access to medicines in New Zealand. BMJ Open. 2012;2(2).
46. Kelsey J. Hidden agendas what we need to know about the TPPA. BWB texts; 2013 [cited. Available from: www.bwb.co.nz.

47. Gallego G, Taylor SJ, Brien JaE. Funding and access to high cost medicines in public hospitals in Australia: Decision-makers' perspectives. Health Policy. 2009;92(1):27-34.
48. Pharmaceutical Management Agency. NPPA outcome report cumulative for March - December (updated 24/1/13). Pharmaceutical Management Agency,; 2013 [cited]. Available from: http://www.pharmac.health.nz/ckeditor_assets/attachments/249/summary_of_nppa_decisions_dec_12.pdf.
49. Forman J, Taruscio D, Llera VA, Barrera LA, Coté TR, Edfjäll C, et al. The need for worldwide policy and action plans for rare diseases. Acta Paediatrica, International Journal of Paediatrics. 2012;101(8):805-7.
50. Lu CY, Williams K, Day R, et al.Access to high cost drugs in Australia. British Medical Journal. 2004;329(7463):415-6.
51. Lee TH, Emanuel EJ. Tier 4 drugs and the fraying of the social compact. New England Journal of Medicine. 2008;359(4):333-5.
52. Simoens S, Cassiman D, Dooms M, Picavet E. Orphan drugs for rare diseases: Is it time to revisit their special market access status? Drugs. 2012;72(11):1437-43.
53. Munoz J, Kurzrock R. Targeted therapy in rare cancers-adopting the orphans. Nature Reviews Clinical Oncology. 2012;9(11):631-42.
54. Hudson I, Breckenridge A. The challenges of orphan drugs and orphan diseases: Real and imagined. Clinical Pharmacology and Therapeutics. 2012;92(2):151-3.
55. Drummond MF, Wilson DA, Kanavos P, et al. Assessing the economic challenges posed by orphan drugs. International Journal of Technology Assessment in Health Care. 2007;23(1):36-42.
56. Joppi R, Bertele V, Garattini S. Orphan drugs, orphan diseases. The first decade of orphan drug legislation in the EU. European Journal of Clinical Pharmacology. 2013;69(4):1009-24.
57. Largent EA, Pearson SD. Which orphans will find a home? The rule of rescue in resource allocation for rare diseases. Hastings Center Report. 2012;42(1):27-34.
58. Cheema PK, Gavura S, Migus M, et al. International variability in the reimbursement of cancer drugs by publically funded drug programs. Current Oncology. 2012;19(3):e165-e76.
59. Lu CY, Macneill P, Williams K, et al. Access to high cost medicines in Australia: Ethical perspectives. Australia and New Zealand Health Policy. 2008;5.

Table one: Stakeholder Characteristics:

Stakeholder Group	Number (n)	Professional Title/Characteristics (n)
Academia (Ac)	3	Sociologist (2) Pharmacoeconomist (1)
Public Service (PS)	5	Politician (1) Medsafe (1) Policy Analyst (1) DHB Planning (1) Pharmac (1)
Medicine (M)	4	Oncologist (1) General Practice (3)
Pharmacist (Ph)	3	DHB (2) Community based (1)
Pharmaceutical Industry (PI)	2	Manufacturing (1) Representative (1)
Patient Group Representative (PGR)	2	Long Term Conditions (2)
Private Health Care Organisation (PHCO)	1	Medical doctor (1)

Table 2: Summary of issues

General Medicines Policy	Ethnicity	PHARMAC	TPP	HCM
• By whom and how are decisions made? • Poor Medicines Policy awareness • Poor Health Literacy; impacting timing of presentation and medicines adherence • Access to Prescribers; physical, timing and affordability • Socio-economic factors (encompassing rural residents) • Sole Supply; out of stock vulnerability and cost, options for intolerance • Discord in recommendations between PTAC and subcommittees • Access challenges on the ability to pay for litigation; non-medical person then decides access • Lack of health impact monitoring • Need for integrated electronic patient records, prescribing information & PHARMAC schedule • Efficiency is static; needs to move toward increases in therapeutic benefit • Registration, evidence and manufacturing requirements constraining for low demand medicines • Increasing demand and cost of medicines impacting affordability • Need for clinical expertise and New Zealand specific research • Need for better medicines	• Socioeconomic factors • Need to use "Health Equity Assessment Tool" to assess policy & inequities/inequalities • Higher burden of disease for Māori and Pacifica; needing risk factor lens • Lacking proper engagement at times • Cultural competency • Use of alternative medicine • Need to capture ethnicity statistics in new initiatives	• Very powerful position of provision; will they cope with role expansion • Young inexperienced staff and high attrition rate • What health outcomes are being measured • Is the lost health opportunity being measured? • Budget too small; need higher percentage of Health budget; "Vote Health" • Cost driving value & causing delays • Need to move to dynamic efficiency • Need analytical framework to compare all health technologies • Submission process inefficient • Economic evaluation influencing therapeutic value evaluation; need to be separated • Questionable how well health professionals understand pharmacoeconomic modelling • In-house economic variables are not necessarily consistent with standard practice or PHARMAC's requirements of suppliers • Hard for suppliers to understand outcome or evaluation process when variables changed • Website very informative but hard to navigate • Concern with expansion into hospitals & limiting choice in acute care & moribund disease setting • Sustainability of current access with	• Many unfamiliar and sceptical of the benefits and who gets them vs. the trade offs • New Zealand small country that needs trade partners • Where will the financial cost be felt and how will it be dealt with • Will there be an increase in the cost of provision • A lot money being spent on health already and benefits low • Australia lost a lot with their agreement with USA; we should learn from it • America's influence is reducing and other forms or protection may evolve • Patent extensions will delay generic entry and raise costs • Will the pharmaceutical industry have greater influence on supply • Access to new medicines may improve • Sovereignty of choice; will there be increased public appeals & litigation • What does transparency mean and does it "cut both ways" • PHARMAC's monopsony is an anathema to the USA • NZ pharmaceutical representative educating "sister" organisation in NZ system • Once a medicine is registered for use, it can be prescribed; PHARMAC may choose to not	• Special Authority access unnecessary once appropriate prescribing established • Need to differentiate high cost vs. highly specialised need and cost • NPPA access scheme brings equitable access for oncology but too early to assess • NPPA capturing patient sub-type classification • NPPA process inefficient and consuming valuable specialist time • Limits access due to cost; but about collective good and who pays • Access cheaper in other countries? • Pharmaceutical companies have good profit margins • Oncology stigma that everyone dies but differences in survival seen at the margins • Evidence does not meet PHARMAC's evaluation criteria • Constraints of "rule of rescue" vs. utilitarian provision

management		increasing demand • Affordability of a panacea	fund it	

Appendix One

Questionnaire

1. Medicines Policies
   - What contribution medicines make to the health status of New Zealand(ers) ?
   - Awareness of "Medicines New Zealand"; the New Zealand Medicines Strategy ?
   - The impact of Medicines Policy upon access to medicines ?
   - What if anything, could be done to improve Medicines Policy; Why and How?

2. Medicines Access and Inequalities/Inequities on the basis of Ethnicity
   - What is their view of medicines access and inequalities based on; the identified ethnicities ?
   - What if anything, could be done to improve access and inequalities for these ethnicities ?

Note: the ethnicities were described as: Māori, Pacifica, Indian or Asian, or such people where English may not be a first language

3. Pharmac
   - Awareness of Pharmac's pricing policy ?
   - Description of Pharmac's pricing policy ?
   - Awareness of how Pharmac subsidises and funds medicines ?
   - Pharmac's impact upon access ?
   - Opinion of Pharmac's model of pricing in terms of cost effectiveness, cost utility and reference pricing ?
   - How well Pharmac is performing it's role, what impact has it had ?
   - What is the future for Pharmac, in next 5,10,20 years. What could be the likely issues ?
   - What if anything, could be improved in relation to Pharmac ?
   -

4. Transpacific Partnership Agreement (TPP)
   - With the likely TPP agreement with United States of America; what impact will it have on medicines procurement and availability and, why ?
   - What needs to be considered with the TPP and access to medicines ?

5. Accessing and Funding of High Cost Medicines
   - Awareness of the accessing and funding of High Cost Medicines and opinion of the process ?
   - Impact of Medicines Policy upon access to High Cost Medicines ?
   - What improvements could be made in the accessing and funding of High Cost Medicines?

Note: a description of high cost medicines was given, such as; beyond the average person's ability to afford e.g., some oncology and Rare Diseases medicines

6. Supplementary Questions
   - Have the above questions covered Medicines Policy ?
   - Any other aspects of Medicines Policy affecting access, not covered ?
   - Will the current system of medicines access continue, or not ?
   - What is the future for Medicines Policy ?

Anything else to say in relation to Medicines Policy and the accessing of medicines ?

Consolidated criteria for reporting qualitative studies (COREQ): 32-item checklist

Developed from:
Tong A, Sainsbury P, Craig J. Consolidated criteria for reporting qualitative research (COREQ): a 32-item checklist for interviews and focus groups. *International Journal for Quality in Health Care*. 2007. Volume 19, Number 6: pp. 349 – 357

YOU MUST PROVIDE A RESPONSE FOR ALL ITEMS. ENTER N/A IF NOT APPLICABLE

No. Item	Guide questions/description	Reported on Page #
Domain 1: Research team and reflexivity		
Personal Characteristics		
1. Inter viewer/facilitator	Which author/s conducted the inter view or focus group?	Susan Francis
2. Credentials	What were the researcher's credentials? E.g. PhD, MD	RN, PG Dip
3. Occupation	What was their occupation at the time of the study?	Research Assistant
4. Gender	Was the researcher male or female?	Female
5. Experience and training	What experience or training did the researcher have?	Qualitative, NVivo
Relationship with participants		
6. Relationship established	Was a relationship established prior to study commencement?	An email was sent to introduce the objective and scope of the study
7. Participant knowledge of the interviewer	What did the participants know about the researcher? e.g. personal goals, reasons for doing the research	An interest to conduct research on NZ medicines policy issues
8. Interviewer characteristics	What characteristics were reported about the inter viewer/facilitator? e.g. Bias, assumptions, reasons and interests in the research topic	Stakeholder characteristics are described in table 1.
Domain 2: study design		
Theoretical framework		
9. Methodological orientation and Theory	What methodological orientation was stated to underpin the study? e.g. grounded theory, discourse analysis, ethnography, phenomenology, content analysis	Content analysis General inductive approach
Participant selection		
10. Sampling	How were participants selected? e.g. purposive, convenience, consecutive, snowball	Purposive

11. Method of approach	How were participants approached? e.g. face-to-face, telephone, mail, email	Email, face to face, through telephone
12. Sample size	How many participants were in the study?	20
13. Non-participation	How many people refused to participate or dropped out? Reasons?	6
Setting		
14. Setting of data collection	Where was the data collected? e.g. home, clinic, workplace	Workplace, clinic, office
15. Presence of non-participants	Was anyone else present besides the participants and researchers?	No
16. Description of sample	What are the important characteristics of the sample? e.g. demographic data, date	Interviews conducted Dec 2012-March 2013 Fifteen interviews, were conducted face-to-face and five via telephone; due to geographical or time constraints.
Data collection		
17. Interview guide	Were questions, prompts, guides provided by the authors? Was it pilot tested?	No It was pilot tested
18. Repeat interviews	Were repeat inter views carried out? If yes, how many?	No
19. Audio/visual recording	Did the research use audio or visual recording to collect the data?	Audio recording
20. Field notes	Were field notes made during and/or after the inter view or focus group?	Yes
21. Duration	What was the duration of the inter views or focus group?	The median length of interview ranged from 53 – 56 minutes.
22. Data saturation	Was data saturation discussed?	No
23. Transcripts returned	Were transcripts returned to participants for comment and/or correction?	Yes
Domain 3: analysis and findings		
Data analysis		
24. Number of data coders	How many data coders coded the data?	Two
25. Description of the coding tree	Did authors provide a description of the coding tree?	No
26. Derivation of themes	Were themes identified in advance or derived from the data?	Derived from the data
27. Software	What software, if applicable, was used to manage the data?	NVivo
28. Participant checking	Did participants provide feedback on the	No

	findings?	
Reporting		
29. Quotations presented	Were participant quotations presented to illustrate the themes/findings? Was each quotation identified? e.g. participant number	Yes
30. Data and findings consistent	Was there consistency between the data presented and the findings?	Yes
31. Clarity of major themes	Were major themes clearly presented in the findings?	Yes
32. Clarity of minor themes	Is there a description of diverse cases or discussion of minor themes?	Yes (Presented in Table 2)

Once you have completed this checklist, please save a copy and upload it as part of your submission. When requested to do so as part of the upload process, please select the file type: *Checklist*. You will NOT be able to proceed with submission unless the checklist has been uploaded. Please DO NOT include this checklist as part of the main manuscript document. It must be uploaded as a separate file.