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# PHARMAC decision-making on new medicines. A case study

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PHARMAC – or, officially, the Pharmaceutical Management Agency, Te Pātaka Waihoranga[1] – is New Zealand's funder for drugs and devices that are available through the public health system. Founded in 1993 in the full flush of the market-oriented health reforms of that period it has sought to apply the disciplines of price competition to the market for pharmaceuticals.[2] It also, by extension, in effect runs a national formulary since not all medicines that are passed by Medsafe, New Zealand's regulator of therapeutic products, end up being funded by the taxpayer after PHARMAC assessment.[3]

It is in this context that the paper by Sarkisova, Lessing and Stretton is of particular interest: if PHARMAC is not funding every medicine and device entering the market after passing Medsafe scrutiny, then the nature and quality of the decision-making involved in this selection process becomes especially important.[4] Does PHARMAC lay too much emphasis on cost in these deliberations? Does it take too long? Does it fail to consider the full range of available international data? Does it discriminate against so-called 'rare disorders'? These are all criticisms made of the PHARMAC process, and yet such is the convoluted and relatively opaque nature of PHARMAC decision-making that it is hard to address them.

The paper by Sarkisova *et al.* starts us on the way.[4] The subject of this case study is well chosen since it addresses a new class of medicines for the management of type-2 diabetes that became internationally available in 2005 but from which no entities were funded by PHARMAC until 2018. By contrast, Australia funded these medicines under its Pharmaceutical Benefits Scheme pretty swiftly, mostly between five and even 10 years in advance of New Zealand. So, why did it take so long – financial pressure from a limited budget; contract negotiations over price; maybe uncertainty with data (for example, on safety and/or comparative effectiveness and therapeutic benefit)?

In each one of the three groups of entities under consideration it seems that lack of clarity over safety and therapeutic benefit provided the majority of the delay. This in itself is revealing because it suggests that the traditional 'one-stop-shop' of the medicines regulator is no longer sufficient for funding decisions (if it ever was). The level of information required to clear this regulatory hurdle may just not be rigorous enough for funding decisions and detailed clinical application.

For example, one of the GLP-1 analogues – exenatide – was passed by the US Food and Drug Administration (FDA) in 2005 and by Medsafe in 2007, and yet as late as 2012 the American Diabetes Association and European Association for the Study of Diabetes noted a 'paucity of comparative effectiveness research on long-term treatment outcomes'. Similarly, for DPP-4 inhibitors Medsafe registered one of these in 2008 and yet in that same year a Cochrane review stated that 'long-term data especially on cardiovascular outcomes and safety [was] urgently needed before widespread use'. Finally, Medsafe registered a member of the third category of entities – SGLT-2 inhibitors – in 2015, while the FDA issued warnings of far-reaching side-effects for this medicine in 2015, 2017 and 2018.

In the PHARMAC system there are three levels of assessment: the specialist advisory sub-committee that evaluates the detailed evidence (in this case, for diabetes drugs); the Pharmacology and Therapeutics Advisory Committee (PTAC) which considers submissions from the sub-committees; and the board of PHARMAC itself which signs off on the final funding arrangements after economic analyses and negotiations. It is hard to tell from the detail provided in the paper the exact allocation of time between these three stages of the decision-making and funding process, but it does seem as though a good proportion of the time between registration and funding was taken up by professionally

legitimate uncertainty about issues of safety, comparative effectiveness, and details of clinical application – at least until the professional consensus started to firm up once these new medicines had been tested and applied in different settings around the world.

The paper by Sarkisova and colleagues addresses to an extent the issue of delay, a frequent criticism of PHARMAC. How about the potential narrowness of a de facto national formulary in which crucial medicines are just not listed? For example, Medicines New Zealand argues that New Zealand lists only a quarter to a third of new medicines,[5] and cancer drugs are a perennial point of criticism.[6] In both cases Australia is cited as a striking contrast, listing far more new medicines and funding more cancer drugs.

To take cancer drugs, Evans *et al.* looked at the cancer medicines funded in Australia and New Zealand in 2016,[7] the great majority of which were funded in both countries (89 in total), but in addition there were 26 medicines with sufficient clinical information that were funded in Australia but not in New Zealand. They concluded that most of these additional medicines did not deliver clinically meaningful health gains, and furthermore they were very costly. They end by stating that 'selective funding of new medicines that demonstrate clear clinical benefit and that are cost-effective and affordable is the sensible approach'.

How about the critique from Medicines New Zealand that we are not funding enough of the new medicines coming onto the market? It is impossible to do justice to the detail of this argument, but there are questions to be asked about the comparative therapeutic advantage of all new medicines. For example, Hwang *et al.* considered the therapeutic value of new medicines approved by the FDA and by the European Medicines Agency between 2007 and 2017,[8] relying on the assessments of four national agencies (Canada, France, Germany, and Italy) and one non-profit independent (Prescrire). Using these assessments on nearly 600 approvals they found that less than a third were judged to be of high therapeutic value by at least one of the five agencies. They conclude that this suggests 'a widening gap between regulatory approval and the clinical and public health priorities of health systems, payers, and patients after approval'.

Given the recent review of PHARMAC[9] and the health changes in process, we need less heat and more light in the assessment of PHARMAC's performance, and the paper by Sarkisova and colleagues provides us with a useful and enlightening starting point. Among other things it suggests that PHARMAC performs an important function in filling the 'widening gap' identified by Hwang *et al.* between formal regulatory approval and application in the real-world clinical and financial environments of health systems.

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