

## **Submission to the inquiry into improving New Zealand's environment to support innovation through clinical trials.**

To the Health Select Committee

This submission is from John Forman, 228 Tinakori Rd, Thorndon, executive director of NZORD, the New Zealand Organisation for Rare Disorders.

Though this is a late submission, I understand the committee's deliberations are continuing, so I request that the committee accept and consider it. I wish to appear before the committee to speak to this submission.

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### **Introduction**

NZORD is an umbrella group set up to provide information, support and advocacy for patients and families affected by rare disorders, and to assist with the development of support groups for them. We build collaborative relationships with health professionals, health planners, researchers and industry, to improve knowledge about rare diseases, improve clinical care, and develop treatments and cures for rare diseases.

Rare diseases, paradoxically, are a common phenomenon. The collective impact of between 7,000 to 9,000 individual rare diseases is such that they affect around 8% of the entire population. Despite this they often receive little priority in health service planning and delivery.

### **An environment of innovation.**

The committee's use of this phrase is most appropriate. Innovation is a major driver of improvements to health and quality of life for the whole population. However it is also a particularly important factor for rare diseases. Decades ago little could be done about most rare diseases, other than to manage symptoms and offer palliative care. But with the rapid growth of genetic knowledge, coupled with discovery of detailed biological functions of cells, and increased understanding of protein function at a molecular level, there is great progress occurring in understanding the causes and progression of rare diseases. There are many more options arising to intervene with clinical care and development of medicines.

Add in recent developments with transgenics and xenotransplantation as two innovative new ways to develop therapeutic options for diseases, and the now frequent discovery through basic research of the genetic causes of many diseases for which the origin was previously unknown, and it become clear that there has been a new wave in health discoveries in recent times.

### **How New Zealand responds to that.**

The first question that arises is how well our system is geared to supporting this innovation and exploiting the discoveries that arise from it. Our conclusion is that overall we do not do this well. There is a history of only modest levels of investment in basic health research, and there has been limited enthusiasm by governments for many years to try and position New Zealand as an innovator or leader in associated areas such as clinical trials.

This approach can be contrasted with the policy frameworks in the USA and the European Union, where there is considerable effort made to encourage research into all diseases, and

in particular specific efforts to understand and treat rare diseases. Both jurisdictions provide significant support for development of new therapeutic compounds, and there is significant infrastructure to support clinical trials.

The Australian experience appears to be one where there is more explicit consideration to research and drug development, as evidenced by the provisions of their medicine strategy.

By contrast, New Zealand's efforts have been very modest. The ethics application process has been revised to provide for greater efficiency, and work has been done to develop a joint registry with Australia for clinical trials, but these matters alone are insufficient to compensate for the low priority attributed to the broader needs of basic research, drug discovery, or treatment innovation, that has plagued our health system for decades.

### **What priorities have driven New Zealand's policy and investments in the past?**

It seems that priorities in health for many years have been geared towards efficiencies in delivery and management of costs; a prime example of this is Pharmac's successful attempts at keeping medicine costs down. However there is at least anecdotal evidence that the rather narrow set of priorities in health research and cost management, may have had negative impacts on career options and retention of clinicians in our public health system, and on our ability to attract investment in clinical trials.

This side of the equation needs more thought. Opportunities for clinicians to be involved in clinical research as part of their career development and maintenance of profession standards, opportunities for patients to gain early access to the benefits of new therapies, and opportunities to gain investment for clinical trials management, are matters that should be factored in and balanced against current approaches.

On the one hand there is a general policy intent that New Zealand science and research should produce first-class and commercially viable outcomes, yet at the same time, at least as far as medicines are concerned, there seems a clear intention that the benefit of cutting edge medical research is unlikely to be paid for by government for our own population.

The contradictions are obvious and we think it is time for a "both and" rather than an "either or" approach to the twin issues of cost savings in health, and development of and funding of innovative medicines in New Zealand. The choices made should be transparent and explicit and any trade-off in cost savings versus improvements to health care of New Zealanders should be clearly identified, including the apparent delay that occurs in access to a wide range of new medicines in New Zealand, compared to access in other OECD countries.

### **Government relationships with the pharmaceutical industry.**

Anecdotally, there appears to be a history of tension between government and the pharmaceutical industry that may have had a negative impact on investment in clinical trials in New Zealand. We don't know the reasons for and extent of this conflict, though we suggest the committee might like to try and find that out from other submitters.

NZORD's perspective on this issue is that there is a need to be explicit about the gains and losses that might arise from a particular policy approach which impacts on the relationship with industry. They are an important player in the development and production of medicines and ideally there would be a partnership approach that encourages cooperation in the development of innovation in the health sector, and the maintenance of clinical trials expertise, while also acknowledging different interests in price negotiations for medicines that are licensed for use.

## **Approval processes for clinical trials.**

The committee chair has publicly raised the claim by one New Zealand researcher that suggests our ethics approval system provides unreasonable impediments to clinical trials. An article by Australian academics<sup>1</sup> reviewed the ethics committee processes for 5 countries including NZ. In contrast they concluded that researchers in New Zealand tend to talk very positively about our processes, particularly the fact that we have a centralised system that has only one major ethical review process. In fact they specifically mentioned that this was better for the researcher than in other countries that might have multiple review processes prior to proceeding with clinical trials (see Fitzgerald & Phillips, 2006, p. 68).

NZORD has a strong interest in the ethical and safety considerations of all health procedures and clinical trials. Our support for research, innovation, and clinical trials is always modified by the need for very good systems to protect patient interests and guard against negative outcomes. In the absence of any research evidence that we are aware of suggesting there are unreasonable impediments to setting up clinical trials, we would not support any loosening of the current system of ethics approval, though we would support any efficiency gains in the approvals process that might be reasonably achievable.

## **Current level of clinical trials investment in New Zealand.**

The raw ANZCTR data suggest that although New Zealand's population is about 20% of Australia's population we have only 17.8% of the recruitment into clinical trials. So in this sense we are lagging behind Australia. In real terms this means that we need to increase the number of trials that are recruiting from New Zealand by about 57, or an increase of 12% (currently 476 are recruiting from NZ) if we want to have equality with Australia.

But in terms of dollars invested, Australia's clinical trial industry is said to be worth \$450 million compared to New Zealand's less precisely estimated value of \$12 - \$30 million. If we take the best case scenario from these data then we have only 7% of Australia's industry worth, but we should have more like 20% which would be about \$90 million.

However reliable the investment statistics are for clinical trials in New Zealand, it seems clear that we are not getting our fair share of the opportunities, and this bears out anecdotal information obtained from clinical trials researchers who advise there has been a gradual decline in investment in clinical trials here since the late 1990s. Pharmac policies and relations with industry are stated anecdotally as key reasons for this, though there may well be other important factors such as the completion of some large international trials around that time.

## **Professional development and career development opportunities through clinical trial participation.**

There is plenty of anecdote and media commentary about the importance of the opportunity to be involved in clinical trials when enhancing the retention of clinicians in hospital practice in New Zealand. No doubt the committee will be briefed on these issues by others with more direct knowledge, but NZORD is concerned that the current environment relating to innovation, constructive partnerships, research investment and investment in the medicines that result from successful trials, may produce a series of negative factors that make it difficult to retain clinicians in our hospital system.

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<sup>1</sup> Fitzgerald, Maureen H. & Phillips, Paul A. (2006) 'Centralized and Non-Centralized Ethics Review: A Five Nation Study', *Accountability in Research*, 13: 1, 47 — 74

Clinical trials also provide more opportunity for serendipitous discovery within clinical practice, particularly if a specialist is affiliated with clinicians who are running trials there is a higher chance that new and interesting avenues of treatment potentials might emerge – this is the true process of science in action. This is particularly important from the perspective of those with rare disorders as new treatments for these disorders often ‘piggy-back’ off more mainstream trials; this is more likely to occur when the appropriate specialists are talking to each other.

Also from the perspective of specialists, being located within an institution that conducts clinical trials provides another potential avenue to refer patients with conditions that are not responding to available medication, or where they may benefit from a new drug in trial. The literature suggests that this is quite common in oncology for instance – with a definite correlation between participants enrolling in drug trials having been referred by an oncologist following the trial. For instance See Sateren et al. (2006)<sup>2</sup> for research supporting this.

New Zealand has an on-going issue with departing specialists. Research suggests that they are leaving because of the “educational and professional opportunities unavailable at home” (Miller et al, 1998, p. 264)<sup>3</sup>. Increasing clinical trials conducted in association with hospitals is likely to provide more opportunities at home and thus has the potential to help retain the NZ trained cohort. See Miller et al (1998) for a discussion of this.

## **Conclusion.**

The success of any proposals to take advantage of clinical trials as a source of income for New Zealand’s health research institutions will depend on creating an environment that

- Is broad in its acceptance of science and innovation as key drivers of health gains,
- includes the building of respectful partnerships between all relevant stakeholders in health research and health service delivery and funding,
- seeks to maximise the benefits of the clinical trials process for professional and career development of our health workforce
- ensures that patient interests are well considered and protected in the system design and processes.

## **Declaration of interest.**

Since 2008 NZORD has received a sponsorship from a clinical trials management company that offered to support our work in building partnerships to improve clinical care, and research into treatments for rare disorders. The funds are given as a grant to assist our research promotion activities and our participation in conferences and seminars.

Yours sincerely,

John Forman  
Executive Director

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<sup>2</sup> Saterin et al. (2006). How Sociodemographics, Presence of Oncology Specialists, and Hospital Cancer Programs Affect Accrual to Cancer Treatment Trials. *Journal of Clinical Oncology* 20: 2109-2117.

<sup>3</sup> Miller, E. A., Laugesen, M., Lee, S. Y. D., & Mick, S. S. (1998). Emigration of New Zealand and Australian physicians to the United States and the international flow of medical personnel. *Health Policy*, 43(3), 253-270.