Department of Health and Ageing second submission to the Productivity Commission

Impact of Advances in Medical Technology on Healthcare Expenditure

The Australian Government Department of Health and Ageing welcomes the opportunity to provide further input to this discussion.

This submission comprises two components; a number of amendments to the first submission lodged by this Department, and comments relating to assertions made in the Productivity Commission's progress report.

Amendments to the first submission of the Department of Health and Ageing

The following paragraph revisions are necessary due to the release of updated information since the first submission was lodged with the Productivity Commission in February 2005.

Health Risk Factors (page 17)

The existing paragraph should be replaced with the following:

The prevalence of obesity among adults aged 25-64 years has doubled over the last two decades, with around 20% now regarded as obese. Over the same period, numbers of Australians suffering high blood pressure has more than halved, but this still amounted to 3.7 million people over the age of 25 years in 1999-2000. Less than one in five adults smoked daily in 2004, compared with 70% of men and 30% of women in the 1950's. Around 35% of people drink alcohol at levels that risk harm in the short term. The corresponding figure for long term alcohol abuse is 10%. About one in six Australians aged 14 years and over in 2001 reported using an illicit drug during the previous 12 months, however there is no clear trend in overall illicit drug use since 1991.

ICT – requirements for standards (page 22)

It was mentioned in the first submission that proposals for the structure, governance, funding and work program of the new national e-health entity were currently being developed for the consideration of Health Ministers in early 2005. The current situation is as follows:

The National E-Health Transition Authority (NEHTA) reported on progress against its work Program to Australia's Health Ministers on 28 January 2005 and

recommended arrangements for the establishment of NEHTA as a separate legal entity to carry on the work from July 2005.

Health Ministers have agreed to the establishment of the Authority for an initial three year period subject to the outcomes of a review after two years of operation. The Authority would be established as a not-for-profit company limited by guarantee, owned and governed by all state, territory and the Australian governments. A governing board would oversee the work of the Authority, with the Authority reporting to Australian Health Ministers on an annual basis.

The Authority will be responsible for fast-tracking the implementation of the necessary standards and basic infrastructure that will connect information across the Australian health care system.

ICT for Improving Healthcare Communication (page 22)

Existing material in this section should be replaced with the following:

Health*Connect* is an overarching national change management strategy that aims to improve safety and quality in health care by facilitating the availability of a range of standardised electronic health information products and services for health care providers and consumers.

Health*Connect* will be an enabler of reform in health care in Australia, supporting new approaches to health care through improved availability of information. Health*Connect* is not a national technology procurement program.

Health*Connect* will provide a platform for further initiatives in health care, supporting:

- new methods of providing health care (consumer-centred coordinated care pathways, health call centres);
- consumers becoming more involved in decisions about their health (consumer autonomy);
- multilateral health information flows (supporting multidisciplinary care teams);
- electronic decision support (expert systems);
- continuing professional education (e-learning);
- new models of health care financing (e.g. online billing or claiming);
- better health service planning (population health analysis); and
- consumer ability to manage the privacy of their health information and participation in associated services.

The Health*Connect* Implementation Strategy leverages existing projects and infrastructure to achieve short-term results (point-to-point electronic transfer of clinical information between healthcare providers) and drive longer-term outcomes (improved quality and safety of health care for consumers), based on the outputs of the work program of NEHTA. NEHTA is an organisation established and managed jointly by all Australian jurisdictions to accelerate the adoption of e-health, and assess the business case for national investment.

The Australian Government and the Governments of all States and Territories have agreed that cooperation is essential to develop the foundations of e-health. As a result, NEHTA has been established by all jurisdictions as the national vehicle to facilitate this cooperation. NEHTA is jointly funded and governed by all Australia jurisdictions.

NEHTA's work program is focused on e-health informatics standards and integrating infrastructure and includes:

- Developing standards for the exchange of clinical information;
- Enabling the unique identification of patients, providers, products and services;
- Enabling the secure electronic transfer of information across the health sector;
- Providing shared information resources;
- Increasing sectoral efficiency by facilitating reform;
- Establishing enabling processes to manage change; and
- Adopting specifications for a national shared electronic health record.

The Health*Connect* Implementation Strategy is a federal partnership for change incorporating a variety of Australian, State and Territory initiatives and policies and private sector investments that have matured during the project's research and development stage.

The expected benefits of Health*Connect* for consumers include:

- An individual electronic health record available nationally with consumer control over access;
- Information tools for individuals to better manage their own health, including access to their own records; and
- A release from the burden of having to remember and repeat important health history every time a consumer comes into contact with the health system.

Benefits for providers are expected to include:

- Quick, reliable access to a broader range of information;
- A secure means of exchanging information among a patient's care team; and
- A reduction in red tape by providing products and services which allow health care providers to work more efficiently.

The Australian Government has committed $128 million over four years to coordinate a staged national implementation of Health*Connect*. Implementation of Health*Connect* has begun in Tasmania, South Australia, and the Katherine region of the Northern Territory, and discussions on implementation projects are underway with New South Wales, Queensland, Victoria, Western Australia and the ACT.

Comments relating to the Progress Report from the Productivity Commission

Executive Summary

Healthcare expenditure growth will continue to be driven by improvements in medical technology. From a holistic perspective, there is scope for the alleviation of costs since the improved health outcomes which are anticipated will necessitate less need for medical interventions in the future. It is far from inevitable that health expenditure will continue to grow as a percentage of GDP.

In the area of regulation, guidelines are constantly being reviewed and improved to ensure that PBAC and MSAC remain effective in their roles as primary regulators of the safety, efficacy and cost effectiveness of pharmaceutical and medical services products. By adopting such a proactive approach, new challenges such as the regulation of combined technologies will be handled in an effective manner.

Progress is being made in the emerging area of e-health. The recent publication of an evaluation of Medi*Connect* and Health*Connect* emphasised the need already recognised for national implementation of electronic health records to focus as much on stakeholder engagement and change management as on technological challenges. The evaluation also addresses concerns expressed in the Progress Report regarding the need for an integrated cost benefit analysis methodology and improved coordination of trials.

Advances in genetic technologies are instigating improvements in the areas of genetic testing, gene therapy and pharmacogenomics. The costs of these treatments vary considerably, with cost effectiveness criteria being of paramount concern. This theme is also illustrated in the fields of prostheses and diagnostic imaging. We disagree with the Commission's contention that a patient's access to technology is necessarily limited under a capped funding arrangement. Treatments will be available where their effectiveness, compared to other treatments, is in proportion with the higher costs.

The TGA, replacing as it did the previous mixture of federal and state responsibilities with a single coherent entity, ensures all medical devices are subject to an appropriate level of pre-market assessment, commensurate with their risk classification. This philosophy ensures the quality, safety, efficacy and timely availability of such goods. Contrary to the impression stated in the Progress Report, all Health Technology Assessment (HTA) activities are fully cost recovered from industry, without funding assistance from the Australian Government.

The following section identifies specific comments within the Progress Report together with a departmental response.

Health technology assessment: pharmaceuticals

The following additional comments should be noted with regard to Chapter 7:

Page 142 para 2
"As most HTA processes in Australia are delineated by technology type, the evaluation of combined technologies is likely to involve more than one HTA body. This could lead to coordination difficulties, confusion and delays in assessing a new technology…"

Guidelines are currently being developed to identify where combined technologies should be considered by the PBAC or the MSAC.

Page 143 para 5
"Despite the widespread use of complementary medicines in Australia, there is little if any assessment of the clinical or cost effectiveness of these products by existing HTA committees"

Complementary medicines are not precluded from being considered by the PBAC for PBS subsidy. However, if PBS subsidy is sought, these would be subject to the same evidentiary requirements that the PBAC applies when evaluating prescription medicines.

Page 144 para 5
"The Commission has received little public comment on procedural and other issues related to the HTA process for new vaccines and would welcome further information."

The government has in the 2005-06 budget transferred vaccine advisory role of ATAGI for funding under he NIP to the PBAC to ensure that cost-effectiveness become a key consideration in recommending vaccines for funding.

The PBAC will revise its guidelines to assist industry in preparing vaccine submissions. As per usual PBAC approach, this will be done in consultation with key stakeholders.

Page 144 para 1
"…. More than 50 per cent of drugs listed have not been assessed for cost effectiveness ...and of those drugs that have been evaluated, relatively few have been re-assessed by the PBAC..."

The proportion of drugs that have undergone cost-effectiveness review will increase gradually over time as some older drugs are delisted and more drugs are assessed for cost-effectiveness are listed.

The 2005-06 Budget includes a measure for cost-effectiveness review of listed drugs.

Page 145 para 1
"…PBAC discounts other study designs that might be more appropriate than clinical trials for the clinical outcome of interest (sub. 30)."

This is untrue - the PBAC guidelines do not specify any minimum evidentiary requirements or mandate randomised controlled trials (RCTs). In fact, studies other than RCTs form a significant proportion of the evidence presented in submissions to the PBAC. As long as a particular study design is sufficiently justified, PBAC will consider it.

Page 147 para 1
"Medicines Australia stated that disagreement with the PBAC over the choice of comparator is one of the major reasons cited by industry for unsuccessful or delayed PBS submissions."

The Department has evidence to suggest that this is less likely to occur if sponsors take advantage of the Department's offer to consult with the Pharmaceutical Benefits Branch before lodging their submissions.

Page 147 para 2
"Of major concern to industry is that by following the PBAC *Guidelines*, this often results in the selection of a cheaper generic drug as the main comparator."

The PBAC guidelines have less to do with generic or cheaper alternative comparators but more to do with the clinical decision framework relating to how the drug and its comparator are likely to be used.

The PBS may be thought of as "purchasing" health outcomes, rather than drugs. CEA is a technique for decision making at the margin. The PBAC is required to consider the costs and benefits of adopting a new medicine in lieu of an existing drug.

If a health outcome can be obtained for the price of a relatively inexpensive drug, then that is the margin at which the decision must be made. To compare a new drug with a more expensive drug simply to make the assessment more favourable does not allow the PBAC to determine the true difference in costs and benefits at the margin.

Page 149 para 5
"…some participants claimed that the PBAC prefers final outcome measures."

The PBAC does take surrogate endpoints into consideration and accepts cost effectiveness analysis modelling beyond the clinical trial. However, a surrogate endpoint trial should be validated by a clinical endpoint trial if this is feasible. As a result, longer clinical *trials are sometimes required.*

Page 150 para 4
"Since 1999, there has been some reduction in the proportion of cost minimisation analyses and an increase in the proportion of cost effectiveness analyses."
The type of economic evaluation conducted, i.e. cost minimisation, cost effectiveness, etc., is determined by the sponsor, and is generally a reflection of the evidentiary basis of the submission. It is not a matter determined by the PBAC.

Page 151 para 3
"Some participants contended that the PBAC gives little or no weight to indirect benefits.

The PBAC Guidelines provide a discussion of the presentation of indirect benefits. In general it is difficult to measure indirect benefits of certain treatments, such as gains in productivity.

However, the PBAC does consider factors such as reduced hospital costs in its assessment of economic benefits.

The treatment of productivity gains in the PBAC's assessment is consistent with guidelines for pharmacoeconomic analysis published elsewhere (eg by the US Department of Human Services and Health).

"… it would appear that the PBAC takes a relatively narrower analytical perspective' when considering the impact of the drug on the total health care budget."

The PBAC takes the broader societal perspective but places as much weight on indirect benefits as the inherent uncertainty in these estimates demand.

It should be noted that the PBAC must take into account the potential impacts of a new drug on the health budget in making its recommendation. The PBAC is open to new methodological approaches as it recognises that methodologies keep evolving.

Page 152 para 1
"…the PBAC should publicly articulate its threshold for an acceptable cost effectiveness ratio and outline its reasons for setting this threshold.'

It should be noted that the PBAC does not have single threshold it considers to be an "acceptable" cost effectiveness ratio. This is because the assessment of cost effectiveness has a number of dimensions not all of which are quantitative in nature. Some of the factors that PBAC takes into account in its decision making include, but are not limited to;

- the severity of condition treated
- presence of effective alternatives
- ability to target therapy to those likely to benefit most
- uncertainty
- equity
- comparative cost effectiveness
- comparative health gain
- affordability to individual and health care system
- financial implications for PBS and for Government health budgets

Page 153

"… concern about PBAC decision making, to a large degree, could be addressed by improving the transparency of its decision making process and greater disclosure of cost effectiveness data…"

The PBAC is always working towards improving the transparency of its decision making processes. Submissions from sponsors are accepted and assessed on the basis of the PBAC Guidelines developed in consultation with the industry. Efforts by the PBAC towards greater transparency such as publishing the basis for its decisions are currently constrained by the industry in requiring that material presented to PBAC in its submissions be treated as commercial-in-confidence information. The PBAC is however working with the industry to improve transparency as part of the implementation of the provisions of the AUSFTA.

Page 154 para 4

"Some participants contended that these restrictions are a way of managing the cost of the
PBS program and generally apply to high cost drugs."

Restrictions are generally intended to confine the use of a drug to circumstances in which it has been demonstrated to be cost effective, and in which it may be used safely.

Pages 155-156 para 6

"… there are claims that some assessment processes are slow… substantial lags between approval of new high cost drugs by the Therapeutic Goods Administration and approval for the Pharmaceutical Benefits Scheme. …The time taken for new drugs to pass through the HTA process is a partial indicator of the efficiency of the process and is relevant to the objective of timely access to medicines."

Submissions to PBAC are considered within a specified timeframe. An application to the PBAC is considered within 17 weeks of submission and a decision is taken at that time. This compares favourably with the time taken in some other jurisdictions (eg NICE)

The time taken to reach a positive recommendation by the PBAC may reflect the quality of the submission.

Page 156 para 6

"Some participants expressed concern about the reduction in the number of PBAC meetings from four to three a year."

The change from four to three meetings in a year was done in consultation with the industry to increase opportunity for the industry to engage more with the assessment process. It is possible that this could resolve issues earlier and minimise some types of rejections.

Page 156 para 2
"In the case of DoHA, departmental appropriations to program management for Medicare (which includes the ongoing development and maintenance of the PBS and Medicare Benefits Schedule) were about $11 million in 2003-04."

Checking has revealed that the $11 million figure was incorrect and that a more appropriate estimate of the cost associated with PBS listing processes and activities was $13 million.

Page 156 para 3
"Medicines Australia argued that requirements for data are costly in terms of conducting specific trials in Australia to collect data required by the PBAC"

There is no expectation that companies will carry out head-to-head clinical trials in Australia solely for the purpose of evaluation for submissions to PBAC. However, companies often carry out studies to support the estimates in the economic analysis.

Page 157 para 2
"… the PBAC accords a high priority to industry and expert consultation but there is limited public consultation."

The extent to which the PBAC is able to undertake public consultation is once again limited by the industry's insistence that the fact of a submission, as well as its content, be treated as confidential.

Page 160 para 4
"Medicines Australia (2002) argued that there is a lack of separation and independence of the PBS listing process from the policy and budgetary functions of DoHA. It also claimed that the Pharmaceutical Evaluation Section, at least in part, carries out the statutory function of the PBAC."

Medicines Australia provides no evidence to support its claims that the PBAC abdicates its responsibilities to or shares its statutory responsibility with the Pharmaceutical Evaluation Section. The role of the Pharmaceutical Evaluation Section is advisory and the PBAC frequently challenges the advice in the process of reaching its recommendation.

Page 162 para 5
"Under reference pricing, the Australian Government pays a subsidy equal to the lowest priced drug (the reference price) in the therapeutic group. According to Sansom (2004), reference pricing applies to narrowly defined therapeutic groups where the drugs are of similar efficacy and health outcomes. There are currently four classes of drugs subject to this arrangement: ACE inhibitors, H_2 receptor antagonists, HMG CoA reductase inhibitors (statins) and the dihydropyridine calcium channel blockers."

Under reference pricing, for drugs considered by the Pharmaceutical Benefits Advisory Committee to be no worse in terms of safety and efficacy, the lowest priced

brand or drug sets the benchmark price for either the other brands of that drug or the other drugs considered of similar safety and efficacy. There are currently 100 reference priced groups of drugs on the PBS. The four mentioned in the quotation are specially identified groups which are interchangeable at the patient level and where a special category of price premium is allowed, known as Therapeutic Group Premiums. This is only one type of reference price group.

Page 165 para 5-7
"… of the drugs that have been assessed and listed since 1993, it appears that few have been subject to re-assessment of clinical and cost effectiveness by the PBAC..."

Similar comments apply as for those made at page 144, para 1

Page 166 para 4
"TGA and PBAC decisions are both appealable to the Administrative Appeals Tribunal (AAT)."

This is incorrect. As previously mentioned, the PBAC does not make decisions, rather, it makes recommendations.

It is the recommendation process, but not the recommendation itself, which is appealable in the Federal Court under the Administrative Decisions (Judicial Review) Act 1977, not the AAT.

Page 168 Summary and findings
- "The PBAC does not assess all medicines used in hospital settings for clinical and cost effectiveness. This appears to have led to some duplication of HTA effort across and possibly within States.

This is due to the health funding arrangements between State and Federal Governments. Also such key considerations as choice of comparator are likely to differ in the hospital setting. At the moment, the PBS is supposed to cover only drugs used in community and not for inpatient use.

- "Once pharmaceuticals are listed on the PBS, there appears to be no systematic process for re-assessing their clinical and cost effectiveness by the PBAC.

The 2005-06 Budget includes a measure for cost-effectiveness review of listed drugs.

"There appear to be several areas in which pharmaceutical assessment processes potentially could be made more efficient and/or consistent with principles of good regulatory design:
- Unlike some overseas health technology assessment processes, the PBS listing process currently provides little opportunity for consultation with patient groups or the general public.
- "The level of information disclosure by the TGA and PBAC regarding drug evaluations has been generally poor compared with other regulatory processes in Australia. Improved disclosure by the PBAC is expected to result from new arrangements under the Australia-United States Free Trade Agreement.

- “A stated intent of restrictions on PBS listed items is to improve cost effectiveness based on clinical grounds. However, as the deliberations of the PBAC are not public, it is difficult to determine whether it has imposed restrictions on certain drugs for fiscal reasons.

Broader access (beyond what is possible under the AUSFTA) to the information in submission to the PBAC for use by health administrators, researchers, prescribers and patients as occurs in some overseas countries would require Government consideration and the cooperation of the industry.

- “The PBAC appears to give little or no weight to indirect benefits of medicines, such as hospital cost savings and gains in productive capacity. In part, this reflects unresolved issues in measuring benefits.

The PBAC gives as much weight to indirect benefits as is appropriate given the uncertainties in such estimates.

Health technology assessment: procedures, devices and ICT

The following additional comments should be noted with regard to Chapter 8:

Page LVI Preliminary finding 8.3

"Feasibility studies and trials used to evaluate HealthConnect appear to be deficient:
- Costs have been assessed in isolation from the assessment of benefits;
- The examination of benefits has been limited in scope;
- Trials have been uncoordinated; and
- Implementation has preceded successful completion of trials."

Since the progress report from the Productivity Commission was released, the report on the evaluation from the Medi*Connect* Field Test and Health*Connect* Trials has been published, together with the Health*Connect* Legal Issues Report. The Legal Issues report brings together findings about a number of legal questions and issues which have emerged from the Health*Connect* Trial and Medi*Connect* Field Tests. These two reports can be found at: http://www.healthconnect.gov.au/whats_new.htm

The research and development phase of HealthConnect (2001 – 2005) included 'proof of concept' trials, the evaluation of which concluded that the national implementation of electronic health records was not just a technological issue, but should focus on stakeholder engagement and change management. The important role of the Australian Government in facilitating a national approach (including with States and Territories) was recognised in the evaluation.

Following evaluation of the research and development phase of Health*Connect*, a revised implement strategy for Health*Connect* has been developed, based on:

- consultations on the Health*Connect* Business Architecture;
- lessons learnt from Health*Connect* and Medi*Connect* trials;
- review of international approaches to the introduction of electronic health records;
- discussion by the Health*Connect* Board; and
- stakeholder feedback on Health*Connect* documentation.

The revised strategy has been published (http://www.healthconnect.gov.au/whats_new.htm), and will be used:

- by Australian, State and Territory governments to drive implementation;
- by a new Health*Connect* Implementation Steering Committee as the 'road map' to guide decision making and monitor progress;
- by staff in Australian, State and Territory government Departments of Health to align regional initiatives to national interoperability standards;
- as the foundation for establishing agreement amongst relevant parties on implementation principles and plans; and
- to guide development of an implementation compliance framework.

The initial implementations of Health*Connect* will commence from July 2005 in the Katherine region of the Northern Territory, South Australia and Tasmania.

The HealthConnect Implementation Strategy focuses on a partnership approach to change management through leveraging existing technology investments in the public and private sectors, rather than a national technology procurement process. Each jurisdiction or private sector organisation investing in components that will be leveraged for Health*Connect* is able to make its own assessment of return on that investment, noting that the cost-benefit assessment is therefore not simply an assessment of the sum of the parts.

The Department of Health and Ageing continues to monitor the international literature on cost-benefit analyses in relation to e-health investment.

Future advances in medical technology

The following additional comments should be noted with regard to Chapter 9:

Impact of Genetic Technologies

Over the next five to ten years, advances in genetic technology are likely to provide significant benefits to the Australian healthcare system. These include improved accuracy of diagnosis, increased efficacy of drug prescribing, and novel avenues for therapeutics. However, a number of applications may involve initial increases in healthcare expenditure. It is anticipated that these costs would be mitigated by the resulting improvements in Australian health and consequent downstream healthcare savings. Because many of the proposed applications of genetics remain largely hypothetical, analysis of possible costs and benefits should be considered with caution.

It is expected that improved knowledge of the human genome will allow better diagnosis of both rare and complex human diseases. Therefore, genetic technologies could lead to an increased level of testing for rare genetic diseases and the introduction of genetic testing as a part of the diagnostic and therapeutic pathway for common complex diseases. An example of this trend is the genetic test for epilepsy that was recently introduced on to the Australian market. The impact of these genetic tests on healthcare expenditure is difficult to generalise. Some tests will largely replace current methods of diagnosis, possibly rendering the technology cost-neutral; others will supplement current methods and therefore probably be additive to the present costs of diagnosis.

Predictive, or presymptomatic, genetic testing is performed on a person who generally has no signs or symptoms of a specific disorder at the time of testing. The test is carried out in order to determine whether or not that person has genetic variations that increase the likelihood that the person may, or will, develop the disorder in the future.[1] Widespread uptake of predictive testing could add costs to the healthcare system. As the person is to all appearances healthy, in the absence of genetic testing they would be unlikely to have had any equivalent test carried out. Further costs may relate to preventative measures. However, it is important to note that these costs would be ameliorated to the extent that preventative measures limit future disease burden. For example, in familial cancer testing, the ability of the individual to undertake preventative measures could distinctly improve future quality of life, and minimise the need for more expensive treatment at a later stage.

Pharmacogenetics refers to the genetic basis for differences in the way that individuals respond to different drugs. It has the capacity to better predict adverse events and improve prescribing efficacy and thereby improve healthcare delivery. Although pharmacogenetics has yet to be broadly incorporated into clinical use, it has been estimated that it could have an impact on the care of more than 15 per cent of patients within 15 years.[2]

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[1] Australian Law Reform Commission: 'Essentially Yours: The protection of human genetic information in Australia' (March 2003), 323.

[2] Melzer D et al, "My very own medicine: what must I know: Information policy for pharmacogenetics" University of Cambridge, A report for the Wellcome Trust (July 2003), 4.

At this stage, it is almost impossible to predict the overall impact that pharmacogenetics will have on healthcare expenditure. Pharmacogenetics will result in increased links between the diagnostic and therapeutic pathway. This may lead to increased expenditure as an additional genetic test will be added to the diagnostic/therapeutic pathway. Cost effectiveness of specific applications of pharmacogenetics will depend on the:

- clinical consequences that are avoided, and subsequent cost savings generated, through the use of pharmacogenetics;
- difficulty of monitoring drug response using current methods;
- strength of association between genotype and clinical phenotype;
- cost of the genetic test; and
- population frequency of the variant gene.

This costing will vary greatly between pharmacogenetic treatments.

Department of Health and Ageing anticipates that the appropriate government expenditure on these new genetic technologies can largely be determined through the existing Medical Services Advisory Committee (MSAC) and Pharmaceutical Benefits Advisory Committee (PBAC) processes. The Australian Health Ministers' Advisory Council (AHMAC) Advisory Group on Human Gene Patents and Genetic Testing is also looking into potential expenditure issues.

The relationship between advances in genetic technologies and future healthcare expenditure may be influenced by the prevalence of gene patents. These generally provide for limited monopoly over a specific genetic sequence, covering all future applications, such as genetic testing and gene therapy. The more extensive these patents are the greater healthcare expenditure on such genetic applications is likely to be. The Australian Law Reform Commission has recently released a report on gene patenting and human health. [3] The Government has not yet responded to this report.

Genetic Testing, Gene Therapy and Pharmacogenomics

The Department of Health and Ageing notes that in relation to the **Genetic testing, gene therapy and pharmacogenomics section** on page 228 of the progress report, while individually rare, collectively genetic conditions affect a significant proportion of the community, particularly when multifactorial conditions (those with substantial genetic and environmental components in their aetiology) are taken into consideration. It is also important to note that while a particular condition, such as cystic fibrosis, may be rare; it may be common within a particular extended family group.

It should also be noted that in Australia, diagnostic and presymptomatic (susceptibility) testing for cancer-causing genes *currently* accounts for a significant proportion of the workload of clinical geneticists and genetic counsellors - thought to be around one-third.

[3] Australian Law Reform Commission: 'Genes and Ingenuity: Gene patenting and human health' (June 2004).

The Institute for the Future (IFF) is based in the United States, and aims to identify trends which will transform the global marketplace. Its work is not necessarily relevant to the Australian context, particularly with regard to the use of genetic technology and the delivery of genetic testing and services. The reports of the IFF have been drawn on heavily by the Productivity Commission, and the Department of Health and Ageing considers that it would have been more appropriate to use Australian sources instead, such as:

- National Health and Medical Research Council (www.nhmrc.gov.au)
- Australian Law Reform Commission (www.alrc.gov.au)
- Human Genetics Society of Australasia (www.hgsa.com.au)
- Association of Genetic Support of Australasia
- State genetic services eg. NSW Genetics Education (www.genetics.com.au) and Genetic Health Services Victoria (www.geneticheathvic.net.au).

The report should present gene names in italics (*BRCA1* and *BRCA2*). *BRCA1* and *BRCA2* genes are responsible for only around 10 per cent of all breast cancer. BRCA testing, when provided through the public health system, is only provided to those patients with a strong family history of breast/ovarian cancer. While some private companies do provide genetic testing for some cancers, it should be noted that genetic testing is also available through public laboratories.

A "genetic test for breast cancer" refers to two distinct types of test. In the first instance, a person affected with breast cancer may have a genetic test to identify if their cancer was caused by an inherited mutation and, if so, what that mutation is. The second type is susceptibility or presymptomatic testing, which looks for a mutation (as previously identified in an affected family member) in an unaffected person. Breast cancer testing is not a test *for* cancer per se. It is to determine the underlying cause of existing cancer, or to identify those family members which *may* develop cancer at some point in their lives.

Other Corrections for Page 228 (paragraph 4):

- The word "hereditary" should be inserted before the word "diseases".
- The words "(most of which are quite rare)" should be inserted after "diseases".
- The word "Down's" should be replaced with "Down".
- The words "environmental and" should be inserted before "lifestyle factors".

In relation to page 299 (paragraph 1) the words "through increased surveillance" should be inserted after "earlier interventions". While it may be possible to overstate the predictive ability of some tests, there are some key points which should be observed in relation to this issue. Firstly, predictive tests will only become more accurate with time, as technology and knowledge of genetics continues to develop. Secondly, tests made available through public laboratories must first be assessed to meet standards for accuracy and sensitivity. Thirdly, while accurate prediction for diseases with complex genetic and environmental aetiology may be difficult, genetics health professionals in Australia are careful to explain to patients that predictive tests are only indicative. They can provide a risk figure, but cannot definitively state whether a person will develop cancer or heart disease.

However, where a genetic risk is indicated, however small, this can be an opportunity to counsel patients about some of the environmental risk factors as well, such as diet.

Additionally, genetic risk figures can be extremely useful in familial cancer. For women at high risk of breast cancer for example, available options may include increased surveillance, prophylactic tamoxifen therapy or prophylactic mastectomy.

The discussion of pharmacogenetics on page 299 (paragraph 2) should be read in consultation with the case study of selective serotonin re-uptake inhibitors (SSRIs) presented elsewhere in the Discussion Paper. SSRIs are a possible area for the application of pharmacogenetics, and this is the subject of current research.

Benefits of Genetic Tests

In relation to page 229 (paragraph 3), the potential benefits of genetic tests are significant, and the expertise of genetics health professionals is extremely valuable in assisting patients to weigh up potential benefits and harms. They can assist patients to fully comprehend the implications and limitations of any screening, diagnostic or predictive/presymptomatic test. Even where treatment or cure is currently unavailable, genetic test information may provide patients with many options they would not otherwise have had the opportunity to consider. For example, prenatal testing may allow parents to prepare for the birth of a child with a disability, exploring options for care or respite care, schooling or assessing their finances. Even receiving correct diagnosis for an existing genetic condition can be an enormous relief for patients. Carrier testing can be a valuable component of family planning, as it can identify those couples at high risk of having a child with a particular genetic condition before they become pregnant. This information can allow for consideration of other reproductive options, such as In-Vitro Fertilisation (IVF) or adoption.

Genetic health services, particularly for diagnosis and carrier testing, have existed in Australia for many years, allowing many individuals and families to find answers, and facilitating appropriate medical and community care and support for those affected.

It should be noted that genetic testing is only undertaken where the patient has given written consent, and in the public health system, where information and an appropriate level of counselling has been provided.

Gene Therapy

The Department of Health and Ageing notes that the words "*chemically-based*" on page 229 (paragraph 4) should be replaced with the words "*chemical compounds*". Further, the statement on page 229 (paragraph 4) about the use of proteins in gene therapy is incorrect (as is the original IFF source material). While proteins are increasingly being used as therapeutic agents often in place of conventional pharmaceutical, this is not considered to be gene therapy. Genes are made of DNA, not protein - the DNA codes for the production of the protein. Gene therapy uses DNA (of which genes are composed) as the therapeutic agent, delivered to the site by a viral vector or by another means. We would strongly suggest that information on gene therapy be sourced from the National Health and Medical Research Council (NHMRC), which hosts the Secretariat for the Gene and related Therapies Research Advisory Panel (GTRAP).

The statement on page 229 (paragraph 4) "*These techniques are experimental but could potentially reduce disease prevalence by effectively preventing disease from developing*" is in direct conflict with the statement in the next paragraph that gene therapy is already being used to treat haemophilia. It is correct to state that gene therapy techniques are currently experimental.

The statement made on Page 229 paragraph 5 *"gene therapy is already being used to treat haemophilia (IFF 2001)…"* is untrue. If, as the IFF states, gene therapy is the use of pharmaceutical agents, then it is true that haemophilia is being treated in this way. However, this is not a correct definition of gene therapy, and the IFF appears to be confused on this point. As previously noted, gene therapy is experimental at this stage. At its most advanced, it is the subject of clinical trials. However, it is not available in the clinical setting for treatment.

It should be noted that PSA testing referred to on page 230 (paragraph 1) is not a genetic/DNA test. However, for tests that are genetic, in that they test DNA or detect a condition which is inherited, ensuring that testing is provided by qualified genetic health professionals can help ensure appropriate use of available genetic testing.

On page 230 (paragraph 2) the words "*diseases that **will** afflict*" should be replaced with "*diseases that **may** afflict*". It should be noted that genetic services, including genetic counselling, have been available in Australia, and many other parts of the world, for at least 15-20 years. It is true that patients experience many psychological and emotional effects as a result of learning of a current or possible future condition, and it is the role of genetic counsellors to help them through this. There is also a large network of genetic support groups in Australia to which many people are referred for further support, including practical assistance. It is also important to note that many patients want to know their future disease status, particularly if they have had family members afflicted with the condition.

As explained above, the benefits of genetic testing, for most people who choose to undertake testing, are significant and wide-ranging. The role of genetic health professionals is important here, because they can help ensure that patients have a clear understanding of both the risks and benefits of genetic testing, whether it is diagnostic or predictive, by exploring options and likely consequences of a positive or negative result, before testing is initiated.

Pharmacogenomics

While pharmacogenomics and pharmacogenetics are often used interchangeably, in the Australian Government context pharmacogenetics is the preferred term, as pharmacogenomics generally refers to the study of drug responses across the whole genome, while pharmacogenetics is more specific to individual patients.

Pharmacogenetics is likely to increase the links present between diagnostic tests and therapy. These tests are likely to complement, rather than substitute for, existing diagnostic and prescribing procedures. These linkages will probably necessitate increased cooperation between the MSAC and PBAC evaluation processes.

The rarity of many genetic conditions means that specialisation and cost sharing between the States for genetic tests may be called for. This cooperation is likely to

increase both quality-assurance and cost-effectiveness. However, hurdles of inter-State funding remain. The AHMAC Advisory Group on Human Gene Patents and Genetic Testing is looking into how collaboration may be achieved.

On page 230 (paragraph 5) the Department of Health and Ageing considers that the words "sold to" should be replaced with "prescribed for".

In relation to page 231 (paragraph 6), while most drugs have an initial monopoly position as the result of patenting, more complex biologics, including genetic therapies, may have a stronger monopoly position due to the critical nature of many of the conditions they address, and a more long-term monopoly due to the difficulty in replicating their production.

The Department of Health and Ageing considers that the wording in page 232 (paragraph 1), which states that – "*Amongst comments received from participants, Department of Health and Ageing (sub. 34, p. 26) considered that gene technology, especially as a method for diagnosing disease, 'may be an area of growth in the coming decade'"* should state "*….will most likely be an area of growth in the coming decade*". This is particularly the case where genetics is expanded to include not only inherited disease, but acquired disease such as cancer (using DNA analysis of tumours to characterise cancer to determine prognosis and appropriate treatment) and infectious disease (DNA analysis of samples from patients to identify the infecting organism).

The issues raised on page 232, paragraph 2, were comprehensively examined by the Australian Law Reform Commission (ALRC) and the Australian Health Ethics Committee (AHEC) of the NHMRC, culminating in the 2003 report *Essentially Yours: The Protection of Human Genetic Information in Australia*. The Australian Government is currently preparing its response to the Report. The establishment of an advisory body on human genetics, one of the key recommendations of the Report, has resulted in the announcement in the May 2005 Budget of the establishment of the Human Genetics Advisory Committee. This paragraph also refers to the ALRC 2004 - however, the Department of Health and Ageing considers that this should be ALRC/AHEC 2003.

The Department of Health and Ageing considers that the statement on page 232 (paragraph 2) stating that, "*If consumers are not willing to share their personal information, the ability to match disease profiles with a product and thereby deliver personalized medicines will be difficult, if not impossible (IFF 2001, p. 33*)" is not correct, as it appears to make advances in this technology conditional on consumers sharing their personal information.

The intent of paragraph 3 on Page 232 is unclear. As with the previous comment regarding consumers and the public, this statement could be seen as an attempt to place responsibility for failure to develop genetic technologies on consumers. People should be free to object to a particular technology if they are uncomfortable with it. Please remove the bracketed comment on genetically modified foods, as this is a very different area with very different issues, and does not add value here. Please insert "diagnostics and" between "gene-based" and "therapies"; and "health" between "gene-based" and "technologies".

The statement made in paragraph 3 on page 232, stating, "*For instance, to recoup investments in research and development, companies will want to patent certain genes and gene sequins but this is controversial"* should be clarified. The ALRC released a Report entitled *Genes and Ingenuity: Gene Patenting and Human Health* in 2004. It may be useful for the Productivity Commission to draw on this report to further develop the above statement.

These issues raised in paragraph 4 on page 232 were examined in detail and reported on by the ALRC and AHEC in *Essentially Yours: The Protection of Human Genetic Information in Australia*. The Australian Government's response to this report will be released shortly. The Productivity Commission will need to take into account the Australian Government's response in the final version of the Impacts of Medical Technology document.

Supplementary Chapter 9 comments

Page 214, Box 9.1
"Estimates of the costs and time required to develop new drugs vary. According to the US Food and Drug Administration, it takes an average 8.5 years and costs approximately US$500 million (FDA 2002b). The Pharmaceutical Research and Manufacturers of America (PhRMA) estimates that 10–15 years are required and Medicines Australia (sub. 30) refers to one estimate that places the cost at about US$800 million and other estimates as high as US$1.7 billion."

There could be a more informed public debate on this if the industry would provide transparent or independently verified costs involved in developing new drugs.

Page 220 para 6
"…if drugs are able to replace other treatments, future expenditure on pharmaceuticals as a proportion of all healthcare expenditure may rise rapidly (IFF 2001)."

There is the need for more robust data presented in sponsors' submissions on the relationship between pharmaceutical expenditure and expenditure on other health technologies.

Page 227 para 5
"Vaccines may also pose a challenge for health technology assessment (HTA) processes — as new types of vaccines are expected to primarily deliver benefits in the longer-term, a question arises as to what is the appropriate discount rate to apply to these benefits."

The government has in the 2005-06 budget included vaccine advisory role in the functions of the PBAC to ensure that cost-effectiveness become a key consideration in recommending vaccines for funding. Analysis of what issues arise is at an early stage.

Page 231 para 7
"… the new biotechnology-based therapies — if listed on the PBS — have the potential to increase considerably the already high growth rates of PBS expenditures."

The cost-effectiveness requirement of PBS listing will help ensure that when such biotechnology-based therapies are PBS listed, they offer value for money.

Page 231 para 8
"Targeting using pharmacogenomics means
Cost will be recovered from smaller population of users
More challenge to current HTA processes which require evidence of efficacy based on extensive clinical trials involving large numbers of patients."

The incremental benefits derived from such therapies will need to be very high to justify their cost. The current situation where so called 'innovative' drugs deliver only marginal benefits but are priced highly need to be critically debated.

Prostheses

The cost to Registered Health Benefit Organisations (health funds) of prostheses and medical devices has been growing over the last decade and is recognised by health funds as a significant driver of premium growth. Prior to the most recent year, prostheses benefits paid by health funds have been increasing by around 30 per cent per annum for the last three years, and prostheses benefits paid in 2003-04 totalled over $647 million. More recently, growth rates have been reduced mainly due to the fact that the new arrangements are imminent, and health funds have decided to await the implementation of these new arrangements rather than negotiate higher benefits.

Prostheses benefits now account for 12 per cent of total hospital benefits, up from 1.7 per cent in 1989-90. Current rates of growth in prostheses costs are estimated to result in a 2 per cent growth in premiums each year.

Health funds currently meet 100 per cent of the cost of all surgically implanted prostheses and other medical devices listed on the Government's Prostheses Schedule (Schedule 5 to the determination under paragraph (bj), Schedule 1 of the *National Health Act 1953*). There are few incentives for ensuring value for money in the current arrangements. For example, there is little evidence-based assessment of safety, effectiveness and cost-effectiveness and pricing arrangements are left to individual health funds and suppliers.

The new arrangements, through the Prostheses and Devices Committee (PDC) (supported by Clinical Advisory Groups (CAGs) and Benefit Negotiators (BN)) are aimed at reducing the rate of growth of prostheses expenditure by:

- using assessment of clinical effectiveness (or clinical design attributes), and where appropriate cost-effectiveness; and
- a centralised benefit negotiation process to introduce competitive tension into the market.

The reduction in the rate of growth of prostheses costs will have a beneficial effect on premium levels and reduce the growth in Government outlays through the Government private health insurance rebates. In addition, the new clinical effectiveness evaluation requirement introduces more rigour into the listing process. Prior to this, there was no clinical evaluation for the majority of new products listed on the Schedule, with the only criteria being that they were listed on the Australian Register of Therapeutic Goods (ARTG).

The structure of the PDC allows it to co-opt expertise in specific areas, such as cost effectiveness, and to seek input from other expert bodies such as the MSAC and the National Joint Replacement Registry (NJRR).

Clinical effectiveness and cost effectiveness

The progress report states that unlike MSAC and the PBAC, the new prostheses arrangements do not consider cost effectiveness.

The clinical evaluations to be undertaken in listing products and the competitive negotiation process are expected to lead to reduced growth in benefits paid for prostheses. Patient and clinician choice will be preserved, with at least one product for each procedure related to a Medicare Benefits Schedule item available at no additional cost to the health fund member (no gap). Patients and clinicians will continue to be able to choose the most appropriate prostheses for their needs at 'no gap'.

The Minister for Health and Ageing has advised that cost effectiveness is within the remit of both the CAGs and the PDC to consider and where suppliers have submitted data this has been considered and taken into account by the CAG when establishing product groups. In 2004, the Minister advised key stakeholders that each CAG should place best patient care as its core in addition to considering clinical effectiveness, and where appropriate, cost effectiveness of the products they review.

Cost effectiveness has not been a major focus in the initial transition to the new arrangements because there is limited comparative clinical data available for most surgically implanted prostheses currently listed. The aim of the initial stage of the new arrangements was to set up a base from which cost effectiveness assessment can be applied into the future. To date, the only area where cost effectiveness has entered into consideration is for some cardiac products. While the Cardiac Prostheses CAG did consider cost effectiveness data submitted, this information was not available for consideration by the other three CAGs (hip, knee and intra-ocular lens).

The application of cost effectiveness for prostheses has not been an issue to date due to a number of points including:

- listing on the Prostheses Schedule was virtually automatic with very little evaluation;
- the lack of comparative data;
- little need to generate comparative data; and
- the lack of public data on price and/or benefit levels.

The new prostheses process of forming product groupings and setting benefit levels for products establishes a base to compare future products. Cost effectiveness can be utilised by suppliers to establish higher benefit levels where clinical benefits are demonstrated.

When the new arrangements are fully established, the onus will be on suppliers to demonstrate clinical advantages in order to obtain a higher benefit than those items already listed, and thus cost effectiveness will play a greater role into the future. However, it is recognised that the rigour of evidence used to assess clinical effectiveness and cost effectiveness in the early stages, may not be as accomplished as that recognised by the PBAC and MSAC. This would be a similar situation as the establishment of cost effectiveness in the pharmaceutical arena.

Introduction of Gap Payments

There will be at least one no-gap clinically appropriate and clinically effective product available for every in-hospital Medicare Benefits Schedule (MBS) procedure. The

new arrangements provide that products can be listed with a gap provided that there is at least one clinically effective and appropriate alternative available at no gap.

Expert clinicians have grouped products according to their comparative clinical effectiveness or comparative clinical design attributes, depending on the clinical evidence available. The clinicians have advised that those products listed with a gap, do not have proven additional clinical or design attributes that would justify a higher benefit than the no gap benefit.

The Prostheses Schedule will list products within their clinical groups and provide the minimum and maximum benefit level for each product. This will enable clinicians to advise their patients of possible gaps and also readily see what clinically effective alternatives are available at no gap.

The introduction of gap payments has the following benefits:

- suppliers can continue to have their products listed, and paid for to the level of the no gap benefit, despite not reducing their price to that of clinically equivalent products;
- the products listed at the no gap level are equivalent products as determined by expert clinicians (that is, the CAGs);
- clinicians and patients still have a wide choice of products available (that is, products are still listed rather than being removed from the Schedule);
- the maximum benefit for a product will be determined by the Minister, with the difference between the minimum benefit and maximum benefit being the limit of the gap a patient will have to pay (health funds may decide to cover part of the gap or suppliers may charge a lower amount); and
- it provides a price signal, especially to clinicians and patients.

Format of new Prostheses Schedule

The first Prostheses Schedule under the new arrangements will be released in August 2005, with effect two months later, in October 2005. The two months will provide hospitals and health funds time to develop systems to cover the administrative arrangements associated with the reform. In addition, this will provide clinicians time to undertake procedures already scheduled without adversely impacting patients through a possible unknown gap payment.

The new format will not separate gap and no gap products. Rather, all products in a clinical group will be listed together, in order for clinicians to readily identify what products require a gap payment, and what clinically effective and appropriate alternatives are available at no gap. The minimum and maximum benefit will be listed against each product, easily identifying the gap payable, if required. This will assist clinicians to fully inform their patients.

Diagnostic Imaging

On page XLV in the Overview, the chart in figure 9 does not include, under Medical Procedures, the four diagnostic imaging Memoranda of Understanding (MoU) Management Committees who have the same role as PSTC (Pathology Services) and MBCC (general medical services). These committees are:

- RMC (Radiology Management Committee)
- CIMC (Cardiac Imaging Management Committee)
- NICEC (Nuclear Imaging Consultative and Economics Committee)
- OGMC (Obstetric and Gynaecological Ultrasound Management Committee).

The second dot point in preliminary findings (Page LV1 in Chapter 8) should state " - *Existing MBS procedures are not generally subject to systematic re-assessment for safety, effectiveness and cost-effectiveness. Whilst MSAC can undertake such re-assessments, its focus is on the evaluation of new technologies and listing of new items."* With respect to diagnostic imaging and pathology, MBS items may be reviewed by the relevant MoU Management Committee. While a systematic approach may not be applied, particular procedures identified as problematic do undergo a review. The outcome of these reviews is either an amendment to the service description in the MBS or, if the procedure is no longer applicable or superseded by new technology, deletion of the item. New technologies identified by such a review would be referred to MSAC.

On Page 15 the Market for Medical Technology the third dot point should more accurately state "*The Australian Government also limits access to subsidised MRI and PET services."*

The first sentence in the last paragraph on page 75 is incorrect. There is capacity to increase the cap if a new technology is approved by the MSAC and listed on the MBS. The cap applies to the use of existing technologies.

The second sentence in the last paragraph on page 75 should be amended to, "*The Australian Government and relevant bodies have entered into four Memoranda of Understanding (MoUs) for Medicare-funded diagnostic imaging services (radiology, cardiac imaging, nuclear medicine and obstetric and gynaecological ultrasound) as a mechanism to promote access to quality and affordable services for patients. Collectively they ensure that spending on diagnostic imaging services will remain within defined tolerances for given five-year target periods."*

The first sentence in paragraph 1 also requires amendment. A patient's access to technology is not necessarily limited under a capped funding arrangement. While the MoUs cap the funding for diagnostic imaging services in the Diagnostic Imaging Services Table of the MBS, patient access to these services has not necessarily been restricted as a result (page 76 – top of page). One of the key features of the MoUs is that there is a strong emphasis on providing greater access to quality diagnostic services at an affordable cost to patients. Where new technologies are approved for funding under Medicare, funding under the MoUs may be adjusted accordingly to ensure patient access to these technologies.

In relation to page 221 (dot point 7), the Government is still to assess the effectiveness and cost-effectiveness of Positron Emission Tomography (PET) in patient management.

In relation to dot point 8, both PET and the fusion of PET and CT are used not only for the diagnosis but also in the management and treatment regime of patients.

In relation to the comment on page 222 (paragraph 3), the Department of Health and Ageing considers that there is currently no evidence on the health benefits to the Australian population for full body computerised tomography (CT) scans. There is however, evidence that the full-body scans lead to a high incidence of "incidental findings". These findings are associated with additional costs for further diagnostic imaging testing and possible invasive procedures. Whole body CT scanning also has a high radiation dose for healthy people. Both local and international experts have consistently not supported the use of whole body CT scanning.

Preliminary conclusions and future policy challenges

The following additional comments should be noted with regard to Chapter 10:

Page 242 para 9
"While there are differences between types of technology that may warrant different treatment, there appears to be scope for a more coordinated and systematic approach across the public and private sectors and across levels of government, especially for medical devices and prostheses."

A more coordinated, consistent application of HTA nationally would require leadership from the Australian Government and cooperation from States and Territories and the private sector.

Comment by the Therapeutic Goods Administration (TGA)

Page XLV Figure 9, repeated page 176 as Figure 8.1
This figure does not accurately reflect the linkages between the TGA and MSAC, whereby MSAC will only review medical devices if they are on the ARTG. There is a need for a direct link between the ARTG (under the heading Devices and Prostheses) and MSAC. Additionally the reference to ODBT needs to be replaced by MDEC (the Medical Devices Evaluation Committee). For the sake of completeness it would also be useful to include a note indicating medical devices are considered by MDEC prior to consideration by DPC and MSAC.

Page 156 Administrative and compliance costs
"In 2003-04, government appropriations to the TGA were about $15 million."

TGA is unsure where this $15 million comes from, though in 2002-03 the government recognised Pan funding of $14.6m which was not actually given to TGA until 2003-04. The Pan recall was a one off event which has nothing to do with regular TGA cost recovery. We believe all reference to this funding should be removed from the report.

Further down the page the report mentions $65 million received by the TGA in 2003-04 "from other sources which is predominantly payments by the drug and device industries for evaluation services and annual charges to maintain listings on the ARTG".

TGA recognised $6.4m in appropriation funding in 2003-04 and this related to one-off funds provided for specific purposes - the Pan recall (another $2.6m), Trans Tasman (2.7m) and interest supplementation ($0.9m). Apart from the interest proceeds, these costs are not related to HTA activities and should be omitted. It would be misleading to retain the reference. For completeness, the report might include a footnote that says something like:
"Although the TGA received $6m of appropriations in 2003-04, these amounts related to once-off specific purposes and were not related to HTA activities, which are fully cost recovered from industry."

It then follows that the statement that the Government spends tens of millions of dollars on HTA activities is false, and would need to be toned down.

Similarly, the report makes an error in reporting income earned from other sources. Revenues from services (cost recovery) totalled $57.8m in 2003-04. TGA also received a residual interest payment ($0.2m), with the remaining income for the year relating to miscellaneous income unrelated to HTA activities, such as laboratory services, internal recharging of administration costs, and overseas training services ($2.5m). The paragraph should be amended to read:
...the TGA received around $58 million in 2003-04 .....

Page 158
References could also be made to the Australia – European Community MRA (EC MRA) on Good Manufacturing Practice (GMP) signed in January 1999 and the

Australia – European Free Trade Association MRA (EFTA MRA) on GMP signed in April 1999.

Page 172
Whilst TGA agree with the conclusion of Section 8.1 that "While useful lessons may be drawn from the experience gained from pharmaceutical assessment, they may not always be directly transferable to the assessment of other technologies," Table 8.1 indicates one of the reasons for this is that the product life cycle for medical devices is relatively short (2-4 years). This may be true for many of the high risk implantable devices (eg: cardiac pacing systems) and device/medicine combination products (eg: drug eluting coronary stents), but there are many devices (eg syringes, bandages, condoms, surgical instruments etc) where the product continues to be supplied with very little change for 10-20 years. We believe this needs to be clarified.

Page 176
TGA suggest the heading 'National Advisory Committees' is amended to 'Agencies and National Advisory Committees. The TGA is not a national advisory committee, it is Australia's regulatory agency. The TGA does seek independent expert advice from ministerially established committees such as Australian Drug Evaluation Committee (ADEC) for pharmaceuticals, Medical Devices Evaluation Committee (MDEC) for medical devices, Medicines Evaluation Committee (MEC) for over the counter medicines and Complementary Medicines Evaluation Committee (CMEC) for complementary medicines.

Page 229
The section on gene therapy should include references to the role of the Gene and related Therapies Research Advisory Panel in approving clinical trials of gene products. The NHMRC may like to provide comment here.

Page 202
The text above Box 8.3 should be amended to read "The product received TGA approval **as a registered device** in June 2002 (box 8.3) following evaluation of pharmaceutical chemistry, toxicology, clinical data, engineering and biomaterials data **by both the TGA's Drug Safety and Evaluation Branch and the Office of Devices Blood and Tissues."**

Within Box 8.3 it should be clarified that in June 2002 "The TGA adds Cypher DES to ARTG as a registered device following a change to the Therapeutic Goods Regulations 1990".

Also with Box 8.3 add "June 2003 Taxus DES registered on ARTG".

Supplementary Comments

When it was introduced in 1991, Australia's regulatory system for therapeutic goods was the first comprehensive national system in Australia, replacing as it did a mixture of federal and state responsibilities. The legislative basis for regulation was the Therapeutic Goods Act 1989 (the Act), which provided for a national and uniform system of control over therapeutic goods in Australia. The basic philosophy of the legislation was to ensure the quality, safety, efficacy and timely availability of such goods.

The system, which at the time was seen as being at the cutting edge of international regulatory practice, contained four key principles:

- creation of a register, the Australian Register of Therapeutic Goods (ARTG), in which all therapeutic goods imported into, supplied within, or exported from Australia had to be included;
- classification of all therapeutic goods into high-risk (registrable), medium-risk (listable) and low-risk (exempt) products. This categorisation of goods, which applied to all therapeutic goods (medicines and devices), determined the degree of pre-market assessment by the TGA prior to inclusion of a product in the ARTG;
- compliance with product standards, as well as labelling and advertising requirements; and
- compliance with manufacturing standards.

'Registrable' goods were subject to detailed pre-market evaluation for quality, safety and efficacy, whereas 'listable' goods were allowed to be supplied following a brief assessment of quality and safety, based on labelling and product information, and the level of compliance with relevant mandatory standards. By default new technologies would fall in the 'listable' category.

Over the ensuing ten years, several deficiencies within the existing legislative framework for devices were identified. The most critical was the inadequacy of the risk-classification process when applied to new device technologies and the consequent sub-optimal pre-market assessment of many clinically important devices. This deficiency resulted in the original 'listing' of cypher DES and the subsequent 'registration' of the product following a change in the Therapeutic Goods Regulations 1990.

In October 2002 the TGA implemented a new risk based regulatory system for medical devices. Key elements of the new system included:

- retention of the ARTG as the central point of control of supply of devices in Australia;
- introduction of new requirements for devices, including:
  - prescribed essential principles for safety and performance for all devices;
  - change from list-based to rules-based risk classification system, with five classes of device - Class I (low risk), Class IIa (low-medium risk), Class IIb (medium-high risk), Class III (high risk) and Active Implantable Medical Device (AIMD);
  - introduction of a choice of procedures that can be employed by manufacturers to demonstrate compliance with Australian regulatory requirements;
  - extension of quality systems requirements to all devices;
  - replacement of unique, mandatory Australian standards with voluntary international standards;
- formation of a new expert committee, the Medical Devices Evaluation Committee, to replace the Therapeutic Devices Evaluation Committee;
- introduction of more powerful mechanisms requiring sponsors to monitor and report device problems, including the introduction of statutory timeframes for reporting problems; *and*
- retention of existing mechanisms of access to unapproved devices.

The new regulatory system for medical devices ensures all medical devices are subject to an appropriate level of pre-market assessment, commensurate with their risk classification. Please contact Rita Maclachlan, Assistant Secretary, Office of

Devices, Blood and Tissues on 02 6232 8700 should you have any queries regarding the TGA.