



Response to ‘Exceptional circumstances and heart transplantation’ letter

Dr Arthur Coverdale has suggested in his letter *Exceptional circumstances and heart transplantation* (NZMJ. 2005;118(1209). URL: <http://www.nzma.org.nz/journal/118-1209/1290>) that both mycophenolate and sirolimus are “first-line” standard treatments for heart transplantation.

Although applications for subsidy for these treatments were initially considered by the Community Exceptional Circumstances (CEC) scheme, it became obvious that this was not the appropriate funding mechanism. CEC was never intended to be used for the provision of “first-line” treatments. CEC has always been about providing funding in truly “exceptional” cases; the definition of “exceptional” generally referring to a national prevalence of less than 10 patients.

Applications to the Exceptional Circumstances Panel for the use of either sirolimus or mycophenolate in heart transplant patients have been made in such numbers that these cases can no longer be considered “exceptional”. PHARMAC and the Exceptional Circumstances Panel wrote to all District Health Board (DHB) transplant groups in both 2003 and 2004 to indicate that the frequency of applications for sirolimus was such that the criteria of rarity could no longer be applied. Instead, as rescue therapy for a functioning transplant is cost-saving to the DHB, the Hospital Exceptional Circumstances (HEC) mechanism could be used. All reasonable requests have been recommended for funding under this mechanism. This funding scheme is more explicit, being a direct cost to the DHB involved and allows funds from the limited CEC budget to be used for other exceptional cases.

Mycophenolate has been considered for listing as first-line therapy for heart transplantation and is in the process of further clinical and economic evaluation. As mycophenolate is much more expensive than its comparator azathioprine, PHARMAC and Pharmacology and Therapeutic Advisory Committee (PTAC) have to ensure that this medicine is both cost-effective as well as safe and efficacious in this role. Factors such as progressive renal impairment, transplant coronary vasculopathy and graft failure, as well as gout, all need to be taken into account, but must be evaluated in properly constituted clinical trials designed to assess these endpoints. Such trials are currently ongoing.

PHARMAC is not able to list an unregistered product or indication in the Pharmaceutical Schedule due to a lack of adequate safety and efficacy data. Sirolimus is not registered for use in heart transplant patients in New Zealand and it therefore cannot be promoted in this context. Indeed, there are important serious adverse effects, including death, that have been associated with this product.

Dr Coverdale also alludes to the Immunosuppressant Subcommittee of PTAC, which has now met and produced guidelines for the use of sirolimus in the New Zealand context. These guidelines will shortly be available.

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