Reimbursement of New High Cost Drugs – Funding the Unfundable?

Expenditure under the Community Drugs Schemes exceeded 1.9 billion in 2008, a five fold increase over the decade 1998-2008. The scheme with the greatest year on year increase in expenditure is the High Tech Drug Scheme accounting for 390 million in 2008. This scheme will come under greater pressure with the advent of very high cost drugs such as eculizumab (Soliris) for the treatment of paroxysmal nocturnal haemoglobinuria (PNH). As such, products cost hundreds of thousands of euro per patient per year (over €350,000 in the case of eculizumab) it is evident that we are facing a new challenge in dealing with very high cost pharmaceuticals entering the Irish market.

An understandable tension may exist between payers responsible for ensuring prudent use of the healthcare budget and pharmaceutical companies and patients who could derive benefit from such high cost products. To date much of the risk in product reimbursement has been borne by the payer (HSE) however the appearance of new, very high cost drugs frequently supported by a weaker evidence base has increased the risk of the reimbursement decisions dramatically, with obvious consequences for decision makers and ultimately taxpayers. How can the HSE possibly reconcile the dilemma of the provision of high cost technologies frequently described as breakthrough in the popular press yet with insufficient evidence to prove cost-effective or value for money? The answer may come from the adoption of schemes that facilitate the availability of high cost drugs to (some) patients whilst limiting the budget impact, focusing the utilisation of these products to situations more likely to prove cost-effective i.e. conditional reimbursement. These various schemes have been described in terms such as performance based or risk sharing schemes.

The schemes may be classified as non-outcomes based or outcomes based. Non outcomes based schemes include price volume agreements e.g. where the payer will specify the level of expenditure and any utilisation above this level will be funded by the manufacturer e.g. a rebate. The payer may also indicate that reimbursement is confined to specific patients, providers or clinical centres aiming to control technology diffusion and reducing the risk of inappropriate use of medicines. Allocation of market share or manufacturer funded treatment initiation are other non outcome based approaches. For those schemes that are outcomes based they may be subdivided into conditional reimbursement (which may be in presence of evidence development or conditional treatment continuation) or reimbursement with outcomes guarantee. A number of excellent publications have appeared in the literature recently. They highlight the distinction of coverage with evidence development where the stated aim is to generate evidence to validate current reimbursement decisions and inform future decisions. The comprehensive review by Stafinski et al indicates that access with evidence development schemes as reimbursement with part payment are mainly applicable to non drug technologies such as diagnostics, surgical and non surgical procedures.

For schemes which are outcomes based, which is the approach most relevant to pharmaceutical products, reimbursement was linked to outcomes guarantee. In the majority of cases (over 80%) this was in the form of health outcomes guaranteed i.e. survival, quality adjusted life year (QALY) or surrogate outcome (change in levels of a known biomarker). In all cases the schemes included uncertainties around clinical effectiveness. The review highlighted four different terms of arrangement. The most frequently used arrangements (over 85%) included where (a) payers provided the drug for a defined period of time, with manufacturers refunding the cost of the drug in patients who did not achieve the targeted health outcome e.g. bortezomib for multiple myeloma - the Valcode Response Scheme in the UK and (b) payers purchasing the drug at half the regular price for the first treatment cycle and then purchasing at full price in those who achieved the targeted outcome and continued on therapy e.g. sorafenib as second line therapy for metastatic renal cell carcinoma in Italy. The remaining options included (c) where payers purchased the drug at full price for a defined period of time, while the manufacturer agreed to lower the price by an amount necessary to ensure cost effectiveness e.g. the Multiple Sclerosis Risk Sharing Scheme in the UK or (d) manufacturers provide the drug at no cost for the first treatment cycle, with payers purchasing the drug for patients who achieved the targeted outcome and continued on treatment. Some of the schemes incorporated a financial outcomes component e.g. ranabizumab (Lucentis) for the treatment of wet age related macular degeneration and lenalidomide (Revlimid) for multiple myeloma in the UK.

It is reasonable to query whether these schemes work or not? From the payers perspective the attractiveness of non outcomes based and outcomes guarantee schemes or a combination of same is evident and an approach which is increasingly used. But what is the evidence with evidence development schemes? Mover & Tunis provide examples of new cost based products that may be considered successful reimbursement with evidence development schemes namely the high dose chemotherapy with autologous bone marrow transplant (HDC-ABMT) study and the National Emphysema Treatment Trial (NETT) in the US contract, the UK Multiple Sclerosis Risk Sharing Scheme is considered by some as an example of a scheme which has failed to deliver (see below) and the recent CRO in the Eculizumab (Soliris) for paroxysmal nocturnal haemoglobinuria (PNH). As such products cost hundreds of thousands of euro per patient per year (over €350,000 in the case of eculizumab) it is evident that we are facing a new challenge in dealing with very high cost pharmaceuticals entering the Irish market.

In relation to pharmacoeconomic we feel decision makers will consider reimbursement with evidence development the exception rather than the rule. In these challenging fiscal times we believe that payers will increasingly turn to the rather less demanding combination of non outcomes based schemes which are less demanding in terms of risk aversion and the increasing importance of budget impact. As we enter the new era of very high cost drugs and the demise of automatic reimbursement it is inevitable that the HSE will enter arrangements along the lines outlined above in an attempt to contain costs whilst enabling patients to gain early access to new high cost drugs.

M Barry, L Tilson
National Centre for Pharmacoeconomics, St James Hospital, James’s St, Dublin 8
Email: mbarry@stjames.ie

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