Accelerated access to medicines
What is it and why is it important?

Jessica Pace, Narcyz Ghinea and Wendy Lipworth

Medicines play a vital role in the treatment of many diseases, and Australian patients generally have access to an excellent range of world-class therapies. However, in recent years, concerns have been raised about the time taken for Australian patients to access new medicines.

Many argue that current regulatory (Therapeutic Goods Administration/TGA) and reimbursement (Pharmaceutical Benefits Advisory Committee/PBAC) processes act as a ‘roadblock’ to access, as they are time-consuming and they demand high levels of evidence of safety, efficacy and cost-effectiveness prior to approval. Concerns about these processes have, in turn, led to calls from both patients and industry for more flexible and streamlined mechanisms for regulating and funding medicines; in other words, for accelerated access to medicines.

The Australian Government has responded to these demands with adjustments to both its regulatory and reimbursement systems. For example, following a recent review, the TGA is planning to introduce a system for expedited approval of medicines within the next two years. And a number of medicines (including those for non-small cell lung cancer and melanoma) have recently been listed on the Pharmaceutical Benefits Scheme (PBS) using the mechanism of ‘managed entry’. This allows for a medicine to be subsidised despite significant uncertainty surrounding its clinical and/or cost-effectiveness. Further data is then collected to resolve this uncertainty and to enable a decision as to whether it will continue to be subsidised (and, if so, at what price).

Australia is not unique in its efforts to expedite access to medicines. Many jurisdictions (including the US, Europe and Japan) have introduced initiatives to streamline the regulatory approval of new medicines, either by increasing the efficiency of regulatory processes or allowing medicines to be approved on the basis of less data than is usually the case. There are also ‘special funds’ in place, both internationally and in Australia, to provide subsidy for medicines that do not meet traditional cost-effectiveness standards for reimbursement, such as UK’s Cancer Drugs Fund and Australia’s Life-Saving Drugs Program.

Providing patients with timely access to innovative therapies is obviously desirable — particularly for life-threatening illnesses or rare diseases for which there are no alternative treatment options. However, it is also important to bear in mind that there is always some degree of uncertainty about the safety and efficacy of new medicines, and this uncertainty is inevitably magnified when regulation and/or subsidisation are accelerated.
This increased uncertainty in turn increases the risk that patients will be exposed to treatments that later prove to be unsafe or ineffective. For example, in 2008 the anticancer agent bevacizumab (Avastin) was granted accelerated approval by the US Food and Drugs Administration (FDA) for the treatment of metastatic breast cancer. However, this approval was revoked in 2011 when further follow-up showed little benefit and several side effects.

While there are pharmacovigilance and post-marketing research processes in place internationally to identify adverse events once medicines are on the market, these systems have serious limitations because they rely on ad hoc adverse event reporting, industry compliance with post-marketing research requirements and the willingness of patients and clinicians to stop using medicines that they might believe to be beneficial. Accelerated access places even more pressure on these processes.

There are also impacts for healthcare resources. When access is accelerated, it is more likely that considerable resources will be devoted to funding medicines that later prove to be unsafe and ineffective and, simultaneously, to treating patients who may have suffered harm after using these. The associated expenses and opportunity costs can be considerable. For example, the UK’s Cancer Drugs Fund exceeded its budget by 50% in 2014 and was converted to a managed entry scheme in 2016. Overall, more than $2 billion was spent to provide access to cancer therapies over the life of the fund. However, as no data was collected on the use of these medicines, we don’t know what impact (if any) this had on patients survival or quality of life or if a larger benefit could have been obtained by spending this money differently.

None of this is to say that accelerated access is necessarily a bad thing. However, we must be alert to the relative lack of evidence available surrounding the safety, effectiveness and cost-effectiveness of medicines made available via such initiatives, and put in place adequate checks and balances to protect both patients and our healthcare system.

“... following a recent review, the TGA is planning to introduce a system for expedited approval of medicines within the next two years.”