

Accessing health outcome data on high-cost medicines in Australia

Christine Y Lu, Kenneth M Williams, Richard O Day

Government-subsidised access to effective medicines in Australia is provided via the Pharmaceutical Benefits Scheme (PBS). Decisions to subsidise medicines are based on assessment of cost-effectiveness (“value for money”) by the Pharmaceutical Benefits Advisory Committee (PBAC).¹ In effect, the PBS is “purchasing” health outcomes. As the majority of medicine use is publicly funded via the PBS, there is an obligation to monitor the outcomes to determine whether the expected health improvements are actually realised.

Monitoring the outcomes of medicine use is a core component of the National Strategy for Quality Use of Medicines,² a pillar of Australia’s National Medicines Policy. Review of the cost-effectiveness of subsidised medicines is also an initiative of the federal government.³ The Drug Utilisation Sub-Committee advises the PBAC on patterns and changes of drug use associated with subsidy restrictions. However, there is minimal evaluation of health outcomes. This article discusses the problem of accessibility to data needed to examine the outcomes and use of high-cost medicines in Australia.

Targeting access to high-cost medicines

The PBS has complex controls to ensure cost-effective use of high-cost medicines by targeting access to subsets of patients. PBS restrictions are based on evidence and a collaborative effort between the PBAC, the respective pharmaceutical companies and representative medical specialists.⁴

Patients must meet criteria for both starting therapy (severe disease inadequately controlled by existing cheaper treatments) and continuing therapy (substantial clinical improvement). Prescribers must provide documentation to support patients’ eligibility. The eligibility of individual patients for initiating or continuing treatment is assessed by Medicare Australia (previously the Health Insurance Commission), which administers the PBS and other health programs. PBS restrictions thus enforce monitoring and documentation of patients’ clinical outcomes from using specialised drugs such as imatinib for treating chronic myeloid leukaemia, and infliximab and etanercept for treating ankylosing spondylitis.

Accessibility to data on the use of biological disease-modifying antirheumatic drugs (bDMARDs: etanercept, infliximab, adalimumab, and anakinra) for treating rheumatoid arthritis provides an illustrative example to raise important issues. Information on the clinical status of each patient that is required as part of an application for bDMARDs (Box 1) could be helpful in evaluating the long-term efficacy and safety of the medicines as well as determining the effectiveness, utility and appropriateness of targeted access schemes. The following are examples of questions that could be answered by researchers with access to such information:

- What are the health outcomes of patients who have commenced, continued, switched between, or withdrawn from biological treatments?
- What are the associations between use of biological agents and rheumatoid factor status, disease duration, and conventional

ABSTRACT

- Data on health outcomes resulting from the use of medicines provide important evidence of cost-effectiveness.
- Currently, clinical information on individual patients, collected by Medicare Australia to assess eligibility for subsidised treatment with high-cost medicines, is inaccessible for research.
- Comprehensive data on drug use and health outcomes should be made accessible, with appropriate regulation, so that the effectiveness, utility and appropriateness of our systems of access to medicines can be independently analysed.
- In the interests of continuous improvement in medical care and optimal use of limited resources, we strongly advocate the enhancement of Medicare Australia databases and liberalisation of arrangements for access to administrative and clinical data.

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antirheumatic drugs usage, and are they predictive of patient response?

- How many rheumatoid-factor-negative patients have been approved to commence biological treatment since the removal of rheumatoid-factor-positive status as an eligibility criterion?
- How does the cost of these medicines compare with the cost savings in other health programs?

Data available on medicine use and health outcomes

Medicare Australia maintains electronic databases of claims from pharmacies for subsidised medicines. The databases also store details of “authority prescription” requests and reimbursements (Box 2, A). These data are potentially accessible at both aggregated and de-identified individual level, including the related prescription claims (medicine usage profile). Requests for access to the data for legitimate research can be made, but the process is slow and difficult. Monthly aggregated prescription volume and expenditure statistics on each PBS item are available in the public domain through Medicare Australia’s website (Box 2, B). While these aggregated data provide some indication of the uptake of bDMARDs,⁵ the actual number of patients using a bDMARD can only be approximated (eg, a crude approximation was that more than 2000 patients had been commenced on bDMARDs by March 2005).⁶ However, the proportion of patients that was approved to continue these medicines cannot be determined. It is also not possible to examine the effects of the “interchangeability rule”, which allows eligible patients to switch between different bDMARDs — an important initiative under the PBS.

We recognise that the primary responsibility of Medicare Australia is to deliver government health programs. On the other hand, its role as a major information source to assist effective

1 Information required by Medicare Australia

For initiating bDMARD therapy

- Patient's Medicare number, name, and dates of previous biological treatment
- History of trialled disease-modifying antirheumatic drugs (name, dosage, duration, reasons for treatment withdrawal)
- Levels of inflammatory markers: erythrocyte sedimentation rate, C-reactive protein
- "Active joint" counts (any joints that are swollen and tender are indicated on a diagram)

For continuing bDMARD therapy

- Patient's Medicare number, name, and dates of previous biological treatment
- Baseline and current levels of inflammatory markers: erythrocyte sedimentation rate, C-reactive protein
- Reduction of "active joint" count (any joints still swollen and tender are indicated on a diagram)

bDMARD = biological disease-modifying antirheumatic drug. ◆

2 Administrative data currently available from Medicare Australia

A. Records related to "authority prescription" requests and claims

- De-identified-patient identifier
- PBS code, generic/brand name and strength of item
- Date authority lodged with Medicare Australia
- Prescriber unique identifier
- Dosage and quantity
- Number of repeats
- Authority reason (eg, "authority only drug"/increased quantity/increased repeats)
- Authority assessment outcome (approved/rejected/pending)
- Officer initials
- State or territory
- Approval number
- Date of approval
- Whether original prescription has been supplied
- Pharmacy identifier
- Date of claim
- Claim identifier
- Whether the claim is for an original or repeat prescription
- Cost reimbursed
- Payment category (general, concession, entitlement)

B. Statistical reports*

- Prescription counts by item
- Cost reimbursed by PBS/RPBS
- Monthly aggregated data (for each state and territory)
- Aggregated data by patient category (general, concession, entitlement)

PBS = Pharmaceutical Benefits Scheme. RPBBS = Repatriation Pharmaceutical Benefits Scheme. *Available at the Medicare Australia website (http://www.medicareaustralia.gov.au/providers/health_statistics/statistical_reporting.htm). ◆

health decision making is becoming increasingly apparent.⁷ The limited usefulness of currently accessible administrative data has been discussed previously.⁸ Our further concern, in the case of high-cost medicines, is that invaluable health information on each individual patient submitted to Medicare Australia (Box 1) is *not* captured in its electronic databases and is, therefore, inaccessible for research purposes. Unfortunately, the information on individual clinical status is regarded as merely supportive of a patient's eligibility for access to high-cost medicines. The existing Medicare Australia databases only capture the "standard" information from an authority prescription (Box 2, A). The value of the clinical information is recognised by Medicare Australia, to the extent that some of this information is recorded manually in an Excel spreadsheet. However, these data do not comply with Medicare Australia's own validated procedures and are not released because the reliability, accuracy and quality of the data are not controlled. This information, which is also required by Medicare Australia's Program Review Division for auditing prescribers, is apparently just as difficult for the Division to obtain.

Data on the use of high-cost medicines managed under the Highly Specialised Drugs Program (eg, infliximab, imatinib) are accessible on request through the Pharmaceutical Access and Quality Branch of the Australian Department of Health and Ageing — but, again, data on health outcomes are unavailable. A recent review of the extent to which information from the PBS and Medicare claims databases can be linked⁹ is a welcome step towards better monitoring of the use of medicines and informing health policies, as well as identifying possible risks and health outcomes resulting from their use.

Comprehensive and comparable datasets on drug usage, clinical details and health outcomes are held by the Department of Veterans' Affairs. The ability to link de-identified datasets is an encouraging example of what can be achieved. These datasets could be used to examine the outcomes of taking specific medicines. However, the uptake of bDMARDs in this special population is low, thereby limiting the usefulness of the data for this drug group. The Department of Health and Ageing, which holds de-identified data from PBS and Medicare claims and performs policy

and program review, could provide useful feedback to prescribers and patients to enhance patient management.

In an attempt to complement the minimal information available through Medicare Australia, rheumatologists have established a voluntary database (the Australian Rheumatology Association Database) to track patient outcomes. However, only about 35% of patients treated with bDMARDs are registered, which may partly reflect the additional administrative burden on practitioners of reporting this information. Some patients do not participate because of the regular questionnaires required and privacy concerns. This emphasises the need for public discussion to address concerns about privacy and to highlight the value of health outcomes research.

The way forward: a need for better access to comprehensive data

Evaluation of drug use and health outcomes can provide important evidence of the effectiveness of medicines in the "real world", where many factors affect the outcome other than the medicines themselves. Analyses of international health outcome data from large observational databases are useful. However, patients treated

with high-cost medicines in Australia are likely to have more severe disease than patients treated with these medicines in other countries, as a result of our generally more restrictive criteria. Therefore, it is important to be able to separately analyse the health outcomes of the Australian patient population.

External, independent examination of the PBS system of access to high-cost medicines from the perspective of system improvement, clinical outcomes or cost-effectiveness is currently impossible. Allowing sufficient access to information that is already collected on drug use and health outcomes should be the basis for improving the quality of the system. We consider that a review and enhancement of Medicare Australia databases, with liberalisation of access to administrative data for approved research, is essential if we are to increase the accountability, transparency, and efficiency of allocating public resources for pharmaceuticals. It would also enhance community confidence in the PBAC's decisions. Better access to accurate, comprehensive and reliable measures of the value of pharmaceutical expenditure and health gains obtained are urgently needed in the interests of better health for all Australians.

Competing interests

Kenneth Williams has been a member of the Advisory Board to the sponsor for adalimumab. Richard Day is a member of the Advisory Board to sponsors for adalimumab, infliximab, and anakinra in Australia. Kenneth Williams and Richard Day have also been contracted to undertake clinical trials of etanercept, infliximab, adalimumab, and anakinra. Recompense for these activities was placed in audited hospital trust funds for use in the research activities of the Department of Clinical Pharmacology and Toxicology, St Vincent's Hospital, Sydney, NSW.

Author details

Christine Y Lu, MSc, PhD Student, Department of Clinical Pharmacology^{1,2}

Kenneth M Williams, PhD, Associate Professor^{1,2}

Richard O Day, MD, FRACP, Professor^{1,2}

1 Faculty of Medicine, University of New South Wales, Sydney, NSW.

2 Department of Clinical Pharmacology and Toxicology, St Vincent's Hospital, Sydney, NSW.

Correspondence: christine.lu@student.unsw.edu.au

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