Managing Medicines Access Programs

Guiding principles for the governance of Medicines Access Programs in Australian hospitals

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Executive Summary

The purpose of these guiding principles is to support Australian public hospitals with the governance of Medicines Access Programs (MAP). The guiding principles are intended to assist drug and therapeutics committees (DTC), health professionals, consumers and pharmaceutical companies with the appropriate implementation, management, delegation of authority, provision of information and oversight of MAP.

MAP are programs offered by pharmaceutical companies (sponsors) to facilitate the deferred cost of, or cost-free or subsidised access to, medicines for hospital patients before the relevant funding arrangements are implemented. These programs include, but are not limited to, compassionate use, expanded access, product familiarisation and cost-share programs.

These guiding principles will facilitate the implementation of consistent good governance and promote the quality use of medicines* within MAP available in Australian public hospitals.

The overarching guiding principles are:

Guiding principle 1. The management and oversight of all MAP should be delegated to a DTC or equivalent.

Guiding principle 2. Appropriate advice regarding the supply of medicines within a MAP should be provided to patients.

Guiding principle 3. Prescribers should comply with DTC requirements when participating in a MAP.

Guiding principle 4. A formal agreement should exist between pharmaceutical companies and hospitals or health service organisations when participating in MAP.

Guiding principle 5. Responsibilities of all parties involved in provision of a MAP should be assigned and clear.

* Quality Use of Medicines (QUM) means judicious selection of treatment options; appropriate choice of medicine when a medicine is required; and safe & effective use of medicines. The National Strategy for Quality Use of Medicines. Canberra: Department of Health and Ageing; 2002.

Overview

Purpose

The purpose of these guiding principles is to provide guidance to hospital prescribers, patients and DTCs on how to access certain medicines (including medicinal products) through Medicines Access Programs (MAP).

Appropriate governance is recommended to ensure patients and hospitals are not unduly exposed to clinical risk of harm (eg, inappropriate discontinuation of therapy) or financial risk of harm (eg, unanticipated costs at the cessation of a MAP).

Medicines should only be supplied for use by hospital clinicians under approved conditions and structures (eg, listed on the relevant formulary, approved on an individual patient basis, or approved as part of a MAP).

Scope

These guiding principles cover all programs offered by pharmaceutical companies to facilitate deferred cost, cost-free or subsidised access to medicines for hospital patients before subsidised listing on the Pharmaceutical Benefits Scheme (PBS), hospital formulary or other relevant funding arrangement.

Such programs include (but are not limited to) Product Familiarisation Program (PFP), Expanded Access Programs (EAP), Compassionate Use, Cost-Share Programs (CSP) and other similarly named access programs. For the purpose of this document, all such programs are collectively referred to as Medicines Access Programs.

This policy does not apply to medicines that are being used as part of a registered clinical trial that has been approved by the relevant human research ethics committee (HREC).

Definitions and terminology

Medicines Access Programs

MAP are programs offered by pharmaceutical companies (sponsors) to facilitate deferred cost, cost-free or subsidised access to medicines for hospital patients before the implementation of relevant funding arrangements.

MAP include, but are not limited to, the following:

Compassionate Use

Compassionate use is a program offered by pharmaceutical companies to provide a medicine free of charge for indications that are not already included in a funded scheme (ie, other MAP arrangement, or eligible clinical trial).

Compassionate use may be determined on an individual patient basis or as part of a wider program. Compassionate use usually involves patients with serious or life-threatening conditions or rescue treatments.

Expanded Access Programs

EAP are programs offered by pharmaceutical companies that provide an investigational product cost-free when associated with participation in a clinical trial.

EAP usually involve patients with serious or life-threatening conditions. An EAP may include patients who do not meet the enrolment criteria for a clinical trial in progress, or those who have been participating in a clinical trial and require continued supply of an investigational product after its conclusion.

Medicines provided under EAP are often not yet registered with the Therapeutic Goods Administration (TGA) for use within Australia.

Product Familiarisation Programs

PFP are programs offered by pharmaceutical companies that are designed to allow the prescriber to evaluate and become familiar with a product while PBS listing is being sought. Products offered under a PFP must be in accordance with the TGA-approved indications and the indication for which PBS listing is being sought.

Cost-Share Programs

CSP are programs offered by pharmaceutical companies that offer a medicine commercially at a reduced price. Use of the product either individually or as a part of a program should be considered as if the drug was simply being marketed at that reduced price.

Treatment costs are shared between a pharmaceutical company and the hospital or health service organisation and/or the patient. Cost-share arrangements may include deferred cost, subsidised supply of a medicine (eg, half price) or arrangements in which supply of a medicine at a reduced price is provided after the purchase of a specified (threshold) amount.

GUIDING PRINCIPLES

The Council of Australian Therapeutic Advisory Groups recommends that the following guiding principles apply to the conduct of MAP within Australian hospitals.

GUIDING PRINCIPLE 1

The management and oversight of all MAP should be delegated to a drug and therapeutics committee or equivalent.

It is recommended that MAP approval be delegated to, and obtained from, a committee (eg, a local/district/state-based DTC) with the required authority included in its remit, before enrolment of any patients in the MAP.

The process for approving a MAP must be well defined to provide transparency, propriety and avoid conflicts of interest. Standard criteria for decision-making should be defined and implemented consistently across all formulary decisions, including those for MAP.

Guiding principle 7 in CATAG's Achieving effective medicines governance. Guiding principles for the roles and responsibilities of drug and therapeutics committees outlines specific considerations. Further information on evaluating appropriateness of use is found in CATAG's Rethinking medicines decision-making in Australian hospitals. Guiding principles for quality use of off-label medicines as well as relevant local policies.

The processes for risk assessment and development and implementation of risk mitigation strategies should be conducted by the committee to ensure no additional risks of harm to the hospital or health service organisation.

A MAP medicine must be used in line with its conditions of approval. Health professionals and patients involved in the process of supply and use must have access to adequate information to support appropriate clinical use of the product.

Health service organisations should implement appropriate administrative arrangements, including the use of a company acknowledgement form, patient consent form and prescriber acknowledgement form (Appendix 1).

All MAP medicines must be stored, managed and dispensed through the hospital pharmacy in accordance with standard procedures applicable to all other medicines. Standard patient co-payments, where applicable, should be levied.

Acceptance of a MAP does not commit any health service organisation, local health network or State/Territory to subsequently place the medicine on its formulary.

The hospital executive, DTC, director of pharmacy or prescriber may decline to participate in a MAP, depending on local circumstances and available resources.

Details of all MAP should be regularly reported to the State or Territory therapeutics advisory group or other nominated agency.

Additional specific requirements may be applied to any MAP arrangement.

Other considerations for DTC management of MAP

A. Patient enrolment in a Product Familiarisation Program

A PFP allows an individual healthcare professional to enrol a maximum of 10 patients in a program in accordance with the Medicines Australia Code of Conduct.

The Medicines Australia Code of Conduct does not specifically limit the number of patients that may participate in other types of MAP, but local governance may limit patient numbers if considered appropriate.

If the sponsor has made an application for PBS or other listing, the PFP must conform to the proposed listing and any restrictions that apply.

B. Considerations for Cost-Share Programs

CSP should not be encouraged. It is preferable that price reductions are negotiated with the supplier via the appropriate procurement process. Negotiations at a jurisdiction level are also preferred to ensure equity of access within the local health system.

CSP requiring initial purchase of product before provision of a cost-free or subsidised supply should not be approved for treatment of patients. If subsidised supply is offered, this must be via a reduced purchase price for the life of the program (eg, 50% cost reduction).

There may be instances when a patient wishes to pay for their own treatment under a CSP. Enrolment of patients in CSP should comply with relevant regulations and policies regarding patient self-funding of medicines.

Even if the patient chooses to wholly self-fund the medicine, the processes for risk assessment and development and implementation of risk mitigation strategies should be conducted by the DTC to ensure no additional risks of harm to the hospital or health service organisation.

GUIDING PRINCIPLE 2

Appropriate advice regarding the supply of medicines within a MAP should be provided to patients.

Patients and their carers must have access to appropriate information to inform their decision to accept a medicine within a MAP arrangement, as they would for any other medicine.

Patients must also be fully informed that the medicine is not routinely available from that hospital and that continuing supply from that institution is dependent on continuance of the MAP at the hospital, and they must agree to treatment under these conditions.

This agreement should be documented in the patient's medical record. A patient consent form (Appendix 1) should be completed and retained in the medical record.

Thereafter, supply through the hospital will be subject to the medicine being listed on the formulary or otherwise approved for use in the hospital.

Ongoing patient management must not be compromised by cessation of a MAP.

If the medicine is not made, or ceases to be, available through the hospital, transfer to another medicine or private supply from another source will be required.

GUIDING PRINCIPLE 3

Prescribers should comply with DTC requirements when participating in a MAP.

Prescribers treating hospital patients must have the approval and support of the hospital DTC and director of pharmacy to participate in a MAP. A prescriber acknowledgement form must be completed and returned to the DTC (Appendix 1).

Prescribers involved with a MAP must declare any actual, potential or perceived conflict of interest to the DTC for each MAP.

Prescribers and other health professionals involved in supply or use of medicines within a MAP must have access to adequate information to support safe and effective use of the medicine and compliance with these guiding principles.

Each individual prescriber must limit their enrolment of patients to a maximum number of 10 patients for each PFP, or as approved locally for other MAP.

Reports at agreed intervals and/or a final report at the end of the MAP and/or when application is made for formulary listing must be submitted to the hospital DTC or delegate.

MAP reports must provide:

- the number of patients enrolled
- details of all adverse events experienced
- effectiveness measures and clinical outcomes of the treatment
- costs of treatment, including associated and incidental costs and cost savings compared with usual treatment
- an assessment by participating prescribers and comments on their experience with the medicine
- the final report must be co-signed by all participating prescribers.

GUIDING PRINCIPLE 4

A formal agreement should exist between pharmaceutical companies and hospital or health service organisations when participating in a MAP.

MAP should be subject to a formal agreement between the hospital and the sponsor supplying the medicine to ensure supply is uninterrupted and free of charge by the sponsor (or as otherwise agreed with the hospital), for as long as the patient's treating prescriber considers there is a clinical benefit, and no equivalent or tolerated therapeutic alternative for the patient remains available in Australia.

The company acknowledgement form (see Appendix 1) should be completed by the pharmaceutical company and returned to the DTC before the medicine is supplied to the patient.

The sponsor should acknowledge that supply to the patient, as indicated above, will continue until the medicine is available through a formal funding mechanism, such as the PBS or the relevant formulary.

MAPs have become increasingly complex, with some demanding the same level of governance and management as a clinical trial. Cost recovery is a management strategy, which can be implemented through a formal agreement. A suggested cost recovery framework is outlined in Appendix 2

GUIDING PRINCIPLE 5

Responsibilities of all parties involved in the provision of the MAP should be assigned and clear.

CATAG recommends that the following responsibilities be assigned to ensure the appropriate conduct of MAP within hospitals and health service organisations.

Appropriate governance and/or oversight should be in place in all facilities. There must be a supportive governance process in place to ensure the following responsibilities are enacted, where applicable. Otherwise, participation in the MAP would not be advised.

PARTY	RESPONSIBILITIES
Senior hospital and health service executives	Ensure all prescribing and pharmacy staff are aware of, and have access to, these guiding principles Ensure the implementation of appropriate governance and administrative arrangements to reflect these guiding principles, including application and completion of pharmaceutical company acknowledgement forms, patient consent forms, and prescriber acknowledgement forms
Drug and therapeutics committees (or equivalent)	Provide appropriate governance for access to, and appropriate use of, medicines via MAP within its jurisdiction, ensuring prescribers follow the approval requirements and recommendations of these guiding principles Approve MAP within their hospital or health service organisation Ensure administrative requirements for MAP are met Ensure that their hospital pharmacy department agrees with and has the resources to support participation in MAP Collect relevant MAP reports from prescribers and forward these to their State or Territory therapeutics advisory group (TAG) (or equivalent). See Appendix 1 for example of registration form
Directors of pharmacy (or delegate)	Ensure medicines used at their hospital under MAP are supplied in accordance with these guiding principles and used in line with the indications specified at the time of a MAP approval and in line with local DTC provisions for information and education of health professionals and patients about appropriate use of the medicine Ensure that their hospital pharmacy department has the resources to support participation in a DTC-approved MAP
Hospital pharmacy departments (or suppliers)	Ensure the storage, management and dispensing of medicines accessed under MAP through the hospital pharmacy are undertaken in accordance with procedures applicable to other medicines, including the provision of adequate information about appropriate use Ensure medicines accessed under MAP and dispensed at their hospital are used in line with the approval for use
Prescribers	Follow the requirements outlined in these guiding principles Inform patients that the medicine is not routinely available from the hospital, and that continuing supply from that institution is dependent on continuance of the MAP at the hospital Obtain the patient's agreement to these conditions before treatment and obtain a signed patient consent form Document the agreement with the patient in the patient's medical record Declare any actual, potential or perceived conflict of interest to the DTC (or equivalent) for each MAP Limit enrolment in PFP to a maximum number of 10 patients (allowing familiarisation with the medicine) and as otherwise DTC-approved for other MAP Submit reports at agreed intervals and/or a final report at the end of the MAP or when application is made for formulary listing to the hospital DTC or delegate Report adverse drug events to the TGA through the Australian Adverse Drugs Reactions System (ADRS) and the hospital DTC (or equivalent) Complete a prescriber acknowledgement form
State or Territory TAG	Provide oversight and governance for access to medicines within its jurisdiction, when applicable Provide a register of MAP in use within their jurisdiction

Appendices

APPENDIX 1: Forms

Examples of a company acknowledgement form, patient consent form and prescriber acknowledgement form can be found on the CATAG website www.catag.org.au

CATAG MAP pharmaceutical company acknowledgement form

CATAG MAP patient consent form

CATAG MAP prescriber acknowledgement form

APPENDIX 2: Cost Recovery Framework

Background

The management of MAP includes the provision of medicines as well as the services and governance related to the judicious, appropriate, effective and safe use of medicines. Management of MAP by healthcare organisations needs to be sustainable in order to ensure the benefits of such programs are realised. Implementation of a cost recovery framework can ensure adequate resources are available for the efficient provision of MAP. A similar framework has been successfully implemented in the provision of clinical trial programs within the public sector.

Cost recovery involves the healthcare organisation charging the non-government sector some or all of the efficient costs of managing a MAP.

The benefits of cost recovery for MAP include:

- Improved sustainability of MAP provision within healthcare organisations
- Improved responsiveness and accountability to MAP stakeholders
- Improved awareness by all stakeholders of the costs entailed in managing MAP
- Improved efficiency and productivity of MAP through workload management
- Facilitated patient access to medicines prior to reimbursement

Cost recovery is intended to assist the healthcare organisation sustainably manage the complexity and risk of making an appropriate medicine, accessible through a MAP, available to patients.

The decision to implement a cost recovery process for a MAP should be assessed on a case by case basis. The relevant Drug and Therapeutics Committee and Pharmacy Department should be involved in such a decision. Cost recovery may cover all or some of the costs of MAP management.

Prior to implementing a cost recovery strategy for a MAP, healthcare organisations should consider the following:

- The costs associated with the management of the MAP including staff costs, training, medicine management processes
- The administration costs associated with cost recovery
- The impact of cost recovery on:
  - The availability of MAP and non-MAP medicines
  - Clinician experience and familiarity with regard to the prescribing, dispensing and administration of the MAP medicine
  - The sustainability of patient treatments
  - Patient experience and familiarity of using the medicine
  - The range of individual patient treatment options

The decision to implement cost recovery should not be mandatory and should be based upon the range of factors described.

Principles for applying a cost recovery framework:

The following principles should be applied to ensure consistent, transparent and accountable charging when applying a cost recovery framework for a MAP:

1. The MAP cost recovery framework promotes the efficient and effective management of the MAP.
   a. MAP administrators/managers should consider whether it is efficient to manage the MAP using a cost recovery framework/strategy. The costs of administering cost recovery should be proportional to the potential revenue from the MAP.
   b. The complexity of the cost recovery schedule should be considered. The more precise and complex a cost recovery framework is, the more expensive its application will be.

2. The MAP cost recovery framework should be transparent and identify accountability

    a. Decisions and key information regarding the MAP should be documented and made available to stakeholders

    b. Financial arrangements should be clearly outlined, included in the MAP submission for DTC approval and adhere to local policies regarding payments from pharmaceutical industry.

    c. The roles and responsibilities of stakeholders throughout the duration of the MAP should be clearly outline and appropriate governance processes put in place.

    d. A well-documented costing schedule should be provided to relevant stakeholders.

3. Ensure good stakeholder engagement

    a. A DTC nominated person should ensure that relevant stakeholders involved in MAP delivery and management should be identified and a communication framework developed to ensure timely consideration and decision-making.

    b. Good stakeholder engagement will result in a better designed, implemented and managed MAP cost recovery processes.

MAP costs recovery fee schedule

A suggested MAP cost recovery fee schedule is outlined in Table 1; Table 1- MAP cost recovery fee schedule.
This incorporates services involving the governance and provision of medicines. The outlined fee schedule is a guide only and can be adapted to suit local requirements. Cost recovery processes for clinical trial programs, which have been in operation for a significant period of time, have informed the development of the MAP cost recovery fee schedule as both programs have similar governance and management processes.

The fee schedule allows individual hospitals and health services to choose from a range of fees, as appropriate to the specific MAP. Regardless of patient numbers each MAP, including compassionate access programs, needs to be assessed for complexity and risk.

CATAG endorses the principle that cost effective medicines should be accessed in public hospitals through DTC-approved formularies. Medicines with insufficient data to determine cost effectiveness should generally be accessed through a clinical trial. Nevertheless, CATAG recognises instances of genuine compassionate access, and suggests cost recovery not be implemented in these cases.

Considerations when higher end fees may be considered:

- Complexity of the MAP: e.g. when multiple dispensing or compounding is required
- Expanded access programs: when there is an extension of a 'clinical trial"
    - Set up fees and governance reviews may be waived if these have been previously undertaken as a 'clinical trial'
- Patient familiarisation programs: intermediate to large size patient populations

APPENDIX 3: Glossary

Adverse drug reaction: a drug response that is noxious and unintended and that occurs at doses normally used or tested in humans for the prophylaxis, diagnosis or therapy of disease, or for the modification of physiological function.[1]

Adverse event: an incident in which harm resulted to a person receiving healthcare.[1]

Conflict of interest: a situation in which an individual or organisation is involved in multiple interests, one of which could possibly corrupt the motivation for an act in the other. Conflict of interest includes situations in which a perceived, potential or actual conflict exists. It is sometimes the perception of a conflict of interest that may be important, whether or not such conflict actually exists, as such perceptions adversely affect relationships within and outside the organisation.

Drug and therapeutics committee (DTC): the group assigned responsibility for governance of the medication management system, and for ensuring the safe and effective use of medicines in the health service organisation.[2] These may also be known as a medicines advisory committee, pharmacy and therapeutics committee, drug committee, drug and therapeutics advisory committee or quality use of medicines committee.

Formulary: a continually updated list of medications and related information, reflecting the clinical judgment of physicians, pharmacists, and other experts in the diagnosis, prophylaxis or treatment of disease and promotion of health. A formulary includes, but is not limited to, a list of medicines and medicine-associated products or devices, medication-use policies, important ancillary drug information, decision-support tools, and organisational guidelines.[3]

Health service organisation: a constituted health service that is responsible for the clinical governance, administration and financial management of one or more service units providing healthcare. A service unit involves a grouping of clinicians and others working in a systematic way to deliver healthcare to patients and can be in any location or setting, including hospital pharmacies, clinics, outpatient facilities, hospitals, practices and clinicians' rooms.[1]

Medicine: a chemical substance given with the intention of preventing, diagnosing, curing, controlling or alleviating disease, or otherwise improving the physical or mental welfare of people. Prescription, non-prescription and complementary medicines, irrespective of their administration route, are included.[1,4]

Patient: a person receiving healthcare. Synonyms for 'patient' include consumer and client.[1]

Pharmaceutical company: organisations supplying medicines, as defined in the *Therapeutic Goods Act 1989*.

Sponsor: a person or company who does one or more of the following:

- exports therapeutic goods from Australia
- imports therapeutic goods into Australia
- manufactures therapeutic goods for supply in Australia or elsewhere
- arranges for another party to import, export or manufacture therapeutic goods.[1]

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3. American Society of Health-System Pharmacists. ASHP guidelines on the pharmacy and therapeutics committee and the formulary system. American Journal of Health-System Pharmacists 2008;65:166-75.
4. Australian Pharmaceutical Advisory Council. Guiding principles for medication management in the community. Canberra: Commonwealth of Australia; 2006.
5. Therapeutic Goods Act 1989. Canberra: Commonwealth of Australia.

APPENDIX 4: How these guiding principles were developed

These guiding principles were developed in consultation and with the agreement of CATAG member organisations listed below:

- ACT Health
- Statewide Therapeutic Drug Committee, (STDC) Tasmania
- NSW Therapeutic Advisory Group (NSW TAG)
- Northern Territory Department of Health
- Queensland Health Medicines Advisory Committee (QHMAC)
- South Australian Medicines Advisory Committee (SAMAC)
- Victorian Therapeutics Advisory Group (Vic TAG)
- Western Australian Therapeutics Advisory Group (WATAG)

During the development of this CATAG document, member organisations undertook consultation, at various stages, with their wider constituents, including hospital DTC, hospital pharmacy departments and clinicians.

DISCLOSURE

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