High-cost medicines: overview of CATAG Guiding Principles and Victoria's current state of play

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Disclosures

- Member, Drug Utilisation Subcommittee of the Pharmaceutical Benefits Advisory Committee (PBAC DUSC)
- Deputy Chair, VicTAG and Victorian representative, CATAG

High-cost medicines

High-cost medicines are:

- Frequently highly impactful in the right situation – the critical intervention
- Often inaccessible by private pathways
- For health services:
  - a variable contributor to budget, often large impact
  - often hard to assess the value of

Current paradigm in Victoria

- For non-PBS medicines, suitability assessed completely at a local health service level alone
- Two main assessment processes often overlap, variably applied
  - Formulary additions
  - Individual patient usage
- Largely reactive, not proactive
- No support for health services, minimal co-ordination
  - Required specialist skills not cultivated or accessible

Difficulties with current paradigm in Victoria

- Frequently ad hoc assessment with variable process
  - Inconsistencies within health services and between health services
  - System not constructed or supported for success

- Result: substantial variation in care, and inherent inefficiency, inequity with suboptimal outcomes for consumers, clinicians, and payers

Navigating high-cost medicines

Guiding principles for the governance of high-cost medicines in Australian hospitals

Expert Advisory Group

- **Professor Catherine Hill (Chair of EAG)** – Director, Rheumatology Unit, The Queen Elizabeth Hospital. Staff Specialist, Royal Adelaide Hospital. Clinical Professor, University of Adelaide, SA.
- **Peter Barclay** – Director of Pharmacy, The Children's Hospital at Westmead, NSW.
- **Dr Sasha Bennett** – Executive Officer, NSW TAG, NSW.
- **Naomi Burgess** – Director, Medicines and Technology Programs (MTP), System Leadership and Design Division, Department for Health and Wellbeing, SA Health, SA.
- **Dr Jonathan Dartnell** – Programs and Clinical Services Manager, NPS MedicineWise, VIC.
- **Catherine Drake** – Society of Hospital Pharmacists of Australia.
- **Associate Professor Tracey-Lea Laba** – NHMRC Early Career (Sidney Sax) Fellow, Centre for Health Economics Research and Evaluation, UTS NSW.
- **Dr David Liew** – Consultant Rheumatologist and Clinical Pharmacologist, Project Lead, Medicines Optimisation Service, Austin Health, VIC.
- **Dr Kylie Mason** – Haematologist, Peter MacCallum Cancer Centre, Melbourne, VIC
- **Terry Melocco** – Director of Pharmacy, St Vincent's Hospital Sydney, NSW.
- **Dr Mary O'Reilly** – Medical Director, Patient Safety & Clinical Excellence, Deputy CMO, Infectious Diseases physician, Austin Health, VIC.
- **Dr Linda Sheahan** – Staff Specialist Palliative Medicine, Clinical Ethics Consultant, Honorary Associate, Centre for Values, Ethics, and the Law in Medicine, University of Sydney, NSW.
- **Dr Kavitha Subramaniam** – Senior Staff Specialist, Gastroenterology and Hepatology Unit, Canberra

CATAG
Council of Australian Therapeutic Advisory Groups

External consultation

- Arthritis Australia
- Australian College of Nursing
- Australian Digital Health Agency
- Australian Medical Association
- Australian Rheumatology Association
- Children's Healthcare Australasia
- Gastroenterological Society of Australia
- Medicines Australia
- Medical Oncology Group of Australia Incorporated (MOGA)
- Northern Territory Government Department of Health
- Pharmaceutical Defence Limited
- Pharmaceutical Society of Australia
- Pricing & PBS Policy Branch, Australian Government Department of Health
- Queensland Government, Department of Health
- Rare Voices Australia
- Royal Australasian College of Medical Administrators
- Society of Hospital Pharmacists Australia
- Tasmanian Government, Department of Health
- Government of South Australia, SA Health
- Therapeutic Goods Administration
- Therapeutic Guidelines
- Western Australia Government, Department of Health.

Conflicts of interest

- Professor Catherine Hill: Member of Pharmaceutical Benefits Advisory Committee (PBAC); Chair, South Australian Medicines Evaluation Panel.
- Dr Tracey-Lea Laba: Member of Drug Utilisation and Economics Sub Committees of PBAC, Member of NSW Statewide Formulary and High Cost Drug Committees.
- Dr David Liew: Member of Drug Utilisation Sub Committee of PBAC.
- Dr Kylie Mason: Member of PBAC; Member of Economics Sub Committee of PBAC.

CATAG Guiding Principles on high-cost medicines

Guiding principle 1. A definition of high-cost medicines should be determined and clearly articulated for use by each medicines governance committee.

Guiding principle 2. Review of high-cost medicines requires members with relevant expertise to facilitate good and effective decision making.

Guiding principle 3. The committee should engage directly with the applicant prior to review to ensure a full understanding of the rationale for the request.

Guiding principle 4. A consistent, robust and transparent procedure for the assessment of high-cost medicine applications should be defined and implemented for use by each medicines governance committee to ensure fair process.

Guiding principle 5. Ethical considerations fundamentally underpin deliberations around high-cost medicines.

Guiding principle 6. The decisions and outcomes of the decision making should be transparent and appropriately communicated to the various audiences.

Guiding principle 7. The high-quality assessment of high-cost medicines requires appropriate training and resourcing.

https://catag.org.au/resource/navigating-high-cost-medicines/

GP1: Clear definitions

Consistent understanding for consistent process

Governance

Guiding principle 1

A definition of high-cost medicines should be determined and clearly articulated for use by each medicines governance committee.

High-cost medicines contribute significantly to hospital expenditures and the higher the cost the greater the implication for the health system. A definition is required to ensure that access to high-cost medicines is managed consistently and fairly using a standardised evidenced-based process.[1] To achieve this, assessment of applications for medicines meeting the criteria for 'high-cost' should be the most comprehensive and rigorous that the resourcing of the DTC allows.

Numerous definitions have been used in Australian medicines governance groups and hospitals to describe a high-cost medicine. Definitions may be described as cost per patient per treatment course or episode of care or a cost per patient per year, or as a finite cost to the health system (whether at a jurisdiction, area or hospital level) per year. Each DTC should determine and approve their own definition of high-cost medicines, contextualised to their local environment for adoption and consistent use.

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When determining t
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- Potential high bu
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- Administration-re
required for med
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- Medicines funded by a pharmaceutical company as part of a Medicines Access Program (if life-long commitment to supply is not contracted with

Table 1: Examples of monetary thresholds for high-cost medicine definitions

ORGANISATION	MONETARY THRESHOLDS
SA Health for high-cost medicine formulary[9]	• ≥$10,000 per patient per treatment course or per year; or • ≥$100,000 for an individual hospital per year; or • ≥$300,000 within the SA public health system per year.
QLD Health high-cost medicines which are not listed on the List of Approved Medicines[10]	• >$10,000 per patient per year/course; or • $100,000 per hospital per year.
NSW TAG[11]	Non-reimbursable acquisition costs equivalent to or more than: A. $1,000 per week per drug per patient, and are used as long-term therapy e.g. for 12 months or longer; or B. $50,000 per treatment course per patient.
Local hospital DTC definition (Sydney Children's Hospital Network)	$15,000 per patient per year, with consideration of impact at that particular hospital.

GP2: Members with relevant expertise

Guiding principle 2

Review of high-cost medicines requires members with relevant expertise to facilitate good and effective decision making.

Broad expertise to deliver rounded and balanced decisions

A collaborative approach for the review and assessment of high-cost medicines, whether formulary or individual use applications, results in a more consistent, rigorous, evidence-based utilisation of these expensive medicines.[1] The review of high-cost medicine applications should involve the engagement of a multidisciplinary group of individuals who have an appropriate skill set and expertise including:

- Clinical specialties
- Medicine evaluation
- Health technology assessment
- Ethics
- Health economics
- Health finance
- Consumers.

It is recommended that a DTC or high-cost medicine subcommittee has a consumer representative as part of their membership to provide a consumer perspective wherever possible to allow for a better evaluation of the balance between potential benefits, costs and possible harms.[12,13] It is essential that the consumer representative understands the need for privacy and confidentiality and declares any conflict of interest as with any other member on the DTC. The patient for which the application is being made should not be the applicant, nor should they be associated in any way with the decision making.

For high-cost items where there is not a DTC member with the relevant expertise, committees should consider seeking other expertise and second opinions from within or outside the health network (e.g. from other states). This can be very beneficial, especially for regional and rural hospitals.

The establishment of a separate committee may be dependent on the setting e.g. state-based versus district- or hospital-based, workload, budget assessment and resource availability.

IPUs may be handled separately to the formulary high-cost medicine committee, depending on the setting and the urgency. Non-urgent IPU applications may be reviewed by the high-cost medicine committee or local DTC.

If establishing a high-cost medicine committee, the goals and scope, governance and reporting arrangements need to be determined at establishment and included in the DTC terms of reference. Particularly important for high-cost medicines is the declaration of conflicts of interest (COI). Participants should declare perceived or actual COI, both pecuniary and non-pecuniary and

GP3: Engage applicant to understand rationale

Application and assessment

Guiding principle 3

The committee should engage directly with the applicant prior to review to ensure a full understanding of the rationale for the request.

DTCs should engage with the applicant, whether for a formulary or individual patient use application, as the critical first step in understanding the application and reviewing any potential variations in practice. Where a comprehensive application has been made, this alone may be adequate engagement. Where the application is lacking in detail, the place in therapy of the requested medicine is unclear, or there is any other uncertainty regarding the application, early engagement with the applicant is essential to ensure that the DTC reaches an appropriate decision. This also results in applicants understanding the shared priorities of the organisation and being cognisant of practical realities. It also provides an opportunity for the applicant to revise and improve their application and provide their opinion and expertise within the context of the therapeutic environment.

Where possible, it may be appropriate for the applicant to be contacted when the application is being considered. This would allow the applicant to answer any questions and help promote efficiencies in the process. The applicant should not be present during any deliberation or discussion.

When possible, gaining the consumer perspective is encouraged, either at the first application or during the consultation process.

Consistently navigating the interface between clinical care and rational evaluation, considering practical realities

GP4: Consistent, robust, transparent procedures

Guiding principle 4

A consistent, robust and transparent procedure for the assessment of high-cost medicine applications should be defined and implemented for use by each medicines governance committee to ensure fair process.

A process that all can understand and rely on, underpinned by assessment with true expertise

A key purpose of the DTC, as per CATAG's *Guiding principles for the roles and responsibilities of Drug and Therapeutics Committees in Australian public hospitals* is maintaining a formulary to ensure the judicious, appropriate, safe, clinically appropriate and cost-effective use of medicines. Without transparent fair process, legitimacy of the process

a. As for all medicines, the decision-making process for high-cost medicines should be defined and transparent, and the grounds for decision making readily available and accessible, to applicants, stakeholders and the community.

b. The financial and economic consequences of funding high-cost medicines are an essential element in the assessment.

c. There should be a standardised process for monitoring of decisions and subsequent outcomes.

d. Applicants should be able to access an appeals process, and the established criteria should be open to revision based on new information including costs information.

GP4: What might assessment look like?

Table 2: Key elements of a high-cost medicine application

APPLICATION TYPE	APPLICATION DETAIL
For all applications	• Indication for therapy (including future use) • Reference to the uniqueness of therapy (no equally safe, effective and practical alternative therapeutic option) • Degree of clinical need of the patient or patient groups (severity/burden of disease/acute or chronic) • Outline previous therapy – is this the 1st/2nd/3rd option, and any alternative treatment pathways • Have all standard therapies been attempted and what are the options if the treatment is not approved? • Evidence to support effectiveness of the medicine • Evidence to support safety and comparative safety of the medicine • Goals of successful therapy and desired outcomes • Pre-defined measures to monitor effectiveness and safety outcomes (e.g. relevant biomarkers, surrogate endpoints or using a quantitative Quality of Life tool) (see section 4c) • Timeline for measurement of outcome(s) • Stopping criteria to indicate when treatment is no longer warranted for the patient • Evidence and approval by the departmental head or other relevant stakeholders with appropriate oversight • Economic evaluations including expected costs and ancillary costs and comparative costs against other available therapies or the current standard of care (see section 4b) • Clinician declaration for any perceived or actual conflicts of interest
For formulary applications	• Engagement with relevant clinicians • A defined patient cohort and criteria for initiating therapy • Anticipated number of patients • Outline if the proposed therapy will supersede other treatment options
For individual patient approval	• Treatment is discussed and agreed to with the patient or care giver (informed patient consent) • Urgency – is the patient's condition life threatening or are there other issues affecting the urgency of the application? • Are there likely to be other similar requests?

Table 3: Example criteria for assessment

EVIDENCE SOURCES	UTILISATION	COSTS
• Evaluations and experiences by other jurisdictions • Ongoing clinical trials/local experience • Guideline recommendations • Current usual care and prognosis • Efficacy and safety based on randomised controlled studies, non-comparative studies, observational studies • Quality appraisal of the strength of evidence	• Population epidemiology – national statistics • Medicine utilisation in own and other jurisdictions • Projected patient utilisation within the jurisdiction initiating and ceasing treatment (steady-state)	• Drug acquisition costs • Storage costs • Drug administration costs • Monitoring costs • Cost off-sets (e.g. reduced Emergency Department presentations) • Adverse event costs • Compounding costs

GP4: Assessment

Notes to Figure 1

A. If this application is a request for a non-urgent IPU the following needs to be considered:

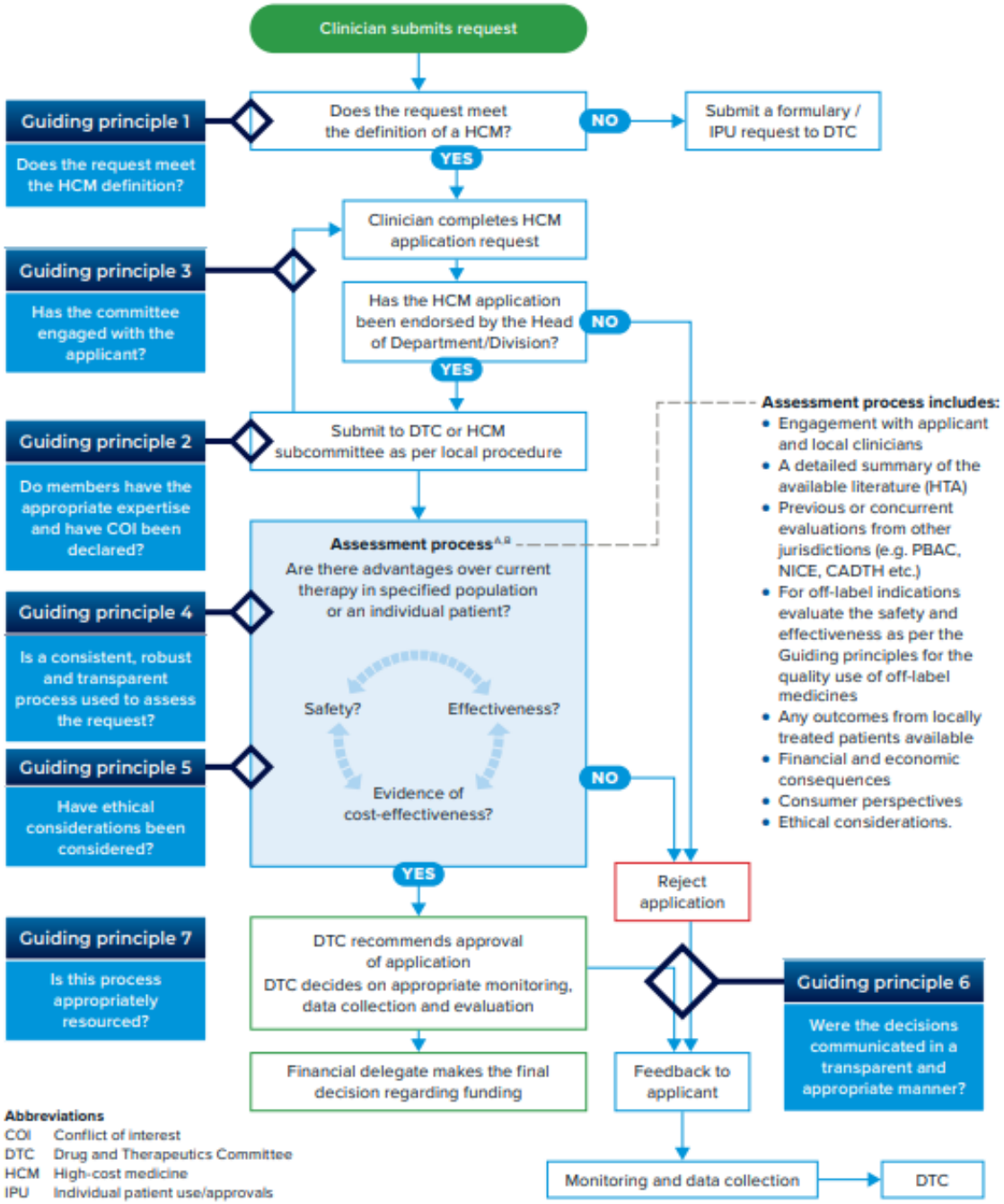
Is there justification for exceptional use?

- serious underlying disease or condition; and
- some evidence to support beneficial effect; and
- potential benefits outweigh potential risks and
- standard therapy has been trialled or is inappropriate; and
- there is written informed consent for medicines that are newly developed, experimental or have known high risk complications or being used off-label or unlicensed use.[27]

If there is no high quality evidence supporting use of a particular medicine, and it is not suitable for 'exceptional indications' or for the purpose of research, use of the medicine is generally not recommended.[28]

B. Consider requests for off-label use using CATAG's *Rethinking Medicines Decision Making: Guiding Principles for the quality use of off-label medicines*, during consideration as a high-cost medicine.

Figure 1: High-cost medicines decision-making algorithm



GP5: Ethical considerations

Guiding principle 5

Ethical considerations fundamentally underpin deliberations around high-cost medicines.

Ethical considerations should be applied to all applications for a high-cost medicine and should consider both the specific individual and the broader community perspective.

Considerations regarding the 'judicious' use of medicines, or 'good' decision making around medicine approvals, are, to some extent, value-based. At the individual application level, the DTC is charged with deliberating on the proposed 'value' of the health outcome, and whether it is thought to be proportional to the cost. This involves the DTC considering, and to some extent agreeing upon, what goals and outcomes they think are worthwhile or important, and making some judgement as to whether the particular high-cost medicine can be considered 'value for money'. This consideration of 'value' must also take into consideration the likelihood of achieving that outcome in an individual patient, or for formulary applications, in a patient group.

Broader ethical considerations relating to fairness are also relevant and include:

- Facilitating access where appropriate, according to due process
- Promoting equity, by considering valid claims for special or differential treatment based on social or economic vulnerability, or those at particular risk of discrimination
- Having an awareness of obligations to resource stewardship, including consideration of sustainability in light of consistency in dealing with subsequent applications
- Considering opportunity cost, and the appropriateness of allocating resources in the high-cost medicine domain as opposed to other areas of healthcare delivery.

Importantly, the DTC should be aware of other broader interests or influences that may affect due

Balancing equity, resource stewardship, opportunity cost
Facilitating access to due process

GP6: Transparency of decisions/outcomes

Communication

Guiding principle 6

The decisions and outcomes of the decision making should be transparent and appropriately communicated to the various audiences.

There are many potential audiences for the process and outcomes of DTC deliberations. In settings where this decision is centralised, the audience may be relatively clear, however this is not always the case. It is important to share the decision with all key stakeholders, including the consumers.

Communication of the decision to the clinician should include:

- Why the particular decision was made, including a statement that the decision was based on cost-effectiveness, safety, budget and equity.
- Feedback that can be provided to the patient(s), which does not damage the relationship between clinician and patient.

The decision (whether for or against) and rationale should also be communicated to the consumers in a timely manner, and be undertaken by an appropriate person (preferably a senior clinician), who can competently explain the complexities of the information in terms the consumer or carer can understand.[13]

The decision should also be documented in medical notes (for individual patient use applications), by the treating clinician, whether approved or rejected.

Clear communication and explanation of rationale, for current and future purposes

GP7: Training and resourcing

Training and resourcing

Guiding principle 7

The high quality assessment of high-cost medicines requires appropriate training and resourcing.

A challenge for the assessment of high-cost medicines is the availability of individuals with appropriate expertise. To facilitate best practice, there is an ongoing need to provide investment in the development of both capacity building and expertise by training those participating in these reviews. When expertise is not available, health service organisations may need to contract external expertise.

Currently there is no standardised training available for DTC members, nor is there a minimum competency criterion required. CATAG recommends all DTC members undertake similar training to assist in 'good decision making', and also work towards a consistent training approach throughout Australia, further contributing to the goal of equitable access. For those reviewing high-cost medicine applications, further training for example in health technology assessment and budget impact analysis is required. It is noted in the report *The New Frontier – Delivering*

It is essential that health service organisations appropriately fund and support DTCs to enable them to fulfil their roles and responsibilities as per CATAG's *Guiding principles for the roles and responsibilities of Drug and Therapeutics Committees in Australian public hospitals*. Positions on DTCs, including the functions of the secretariat, should be funded to account for the expertise, time and effort required to review these applications. CATAG acknowledges that increased resourcing is required to implement these principles.

Creating expertise in assessment, to sustain an informed system

GP: For the future

For the future

Promote equity of access by having a national definition for jurisdictions and hospitals to use for high-cost medicines

One of CATAG's stated objectives is to promote fair process and equity of access for all patients. This requires transparency and standardisation in decision making, evaluation and review between jurisdictions to improve patient centrality within this process.

Medicine access equity means that everyone should have a fair opportunity to access funded medicines to attain their full health potential, and that no one should be disadvantaged from achieving this potential.[26] There are many barriers to equity which include personal and health system barriers. Equitable access is determined by five drivers: medicine availability, accessibility, affordability, acceptability and appropriateness. Medicine availability includes how a medicine is funded and the implementation of those decisions so that everyone who is eligible can access the medicine.[26]

Promote the sharing of assessments and outcome data and the development of a national high-cost medicine formulary

It is important to acknowledge the current duplication of work evaluating medicines, especially high-cost medicines, for availability at Australian public hospitals. This duplication of effort would be increased if budget impact analysis and health technology assessment are introduced for each individual DTC. Along with duplication, this time-intensive process potentially increases time to access of medicines for patients. In situations where patients may be transitioned from one health service to another, for example from a paediatric hospital to adults or metropolitan to regional, the sharing of the assessment and decision making for the high-cost medicine is especially important.

Establishment of national processes is recommended. This could be achieved by formalising and promoting the already established network for sharing resources, through CATAG, whereby

Better collaboration between state jurisdictions, to leverage expertise

Current state of play: straw poll

Which best describes your health service?

Major metropolitan health ser...	10
Specialist hospital	4
Major regional centre	2
Rural or regional health service	3

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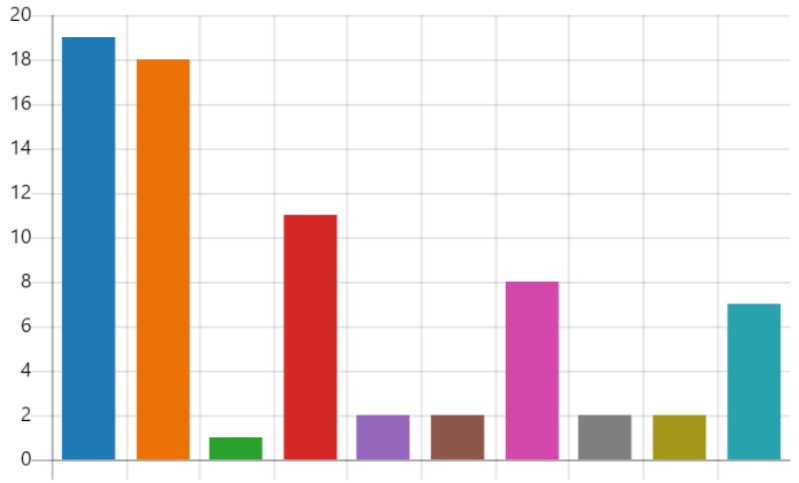
- >$2000 per annum for an IPU
- over 500$
- Over $1000 requires DTC or DTC Executive approval (Pharmacist, CMO, other medical member)
- expenditure is equal to or greater than $1000 net hospital cost per treatment course or per annum
- Greater than $1000
- More than $20,000 per patient per annum
- Cost of therapy > $300 per month
- Thresholds are that requests <$2,500 are approved by DOP (or delegate) between $2,500 and $25,000 are considered by SPU committee and beyond $25,000 need SPU committee review then Exec approval if committee support the request. (High risk medicines may be referred to the SPU committee regardless of cost - eg if condition is common and risk of high expenditure should multiple requests be received)
- >$5000/course or annual treatment
- Greater than $10K for the course of treatment
- Greater than a single treatment costing $1,000
- Thresholds set for DoP, DTC and Executive approvals within DTC ToR
- Medication with course cost above 3 K
- Generally non-formulary and non-PBS medicines that have capacity for budgetary impact as determined by the Pharmacy Management team. These are considered and approved by DTC through one of three mechanisms: assessment of formulary application, individual patient use application for high cost medicines that are not accessible on the PBS, medicines access programs where assurance of ongoing supply is not guaranteed.
- dependant on health fund & whether the patient is a day or overnight patient

Formulary: expertise (GP2)

Which of the following skill sets/expertise do individuals on the group that makes decisions on **Formulary Applications** for high cost medicines possess? (Select all that apply)

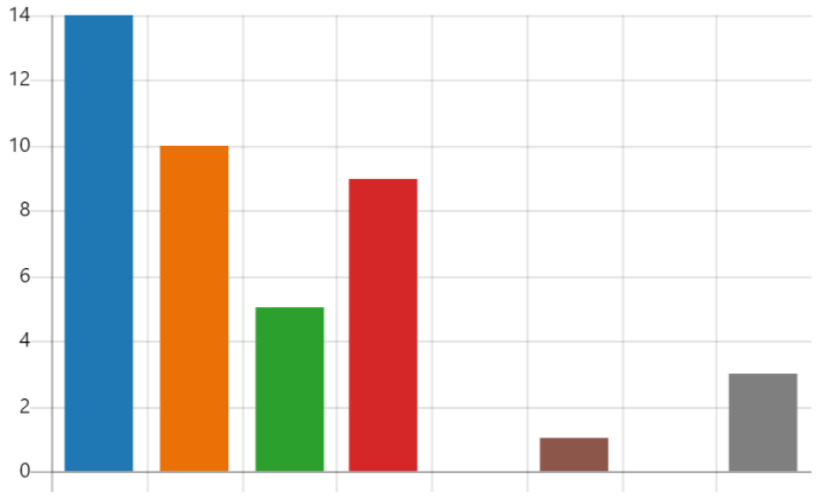
Pharmacist	19
Clinical (medical) specialists	18
Health technology assessment	1
Medicine evaluation	11
Ethics	2
Health economics	2
Health finance	8
Consumers	2
Clinical pharmacologist	2
Other	7



Formulary: engage applicant (GP3)

What support is provided to applicants when submitting a **Formulary Application** for a high cost medicine?
(e.g. is the application screened and/or amended by someone prior to going to the committee/group for approval) Select all that apply.

Director of Pharmacy (or dele...	14
Formulary pharmacist	10
Medical specialist/fellow	5
Head of medical unit submitti...	9
Electronic forcing functions in ...	0
Electronic forcing functions in ...	1
No support provided	0
Other	3



Formulary: algorithm (GP4)

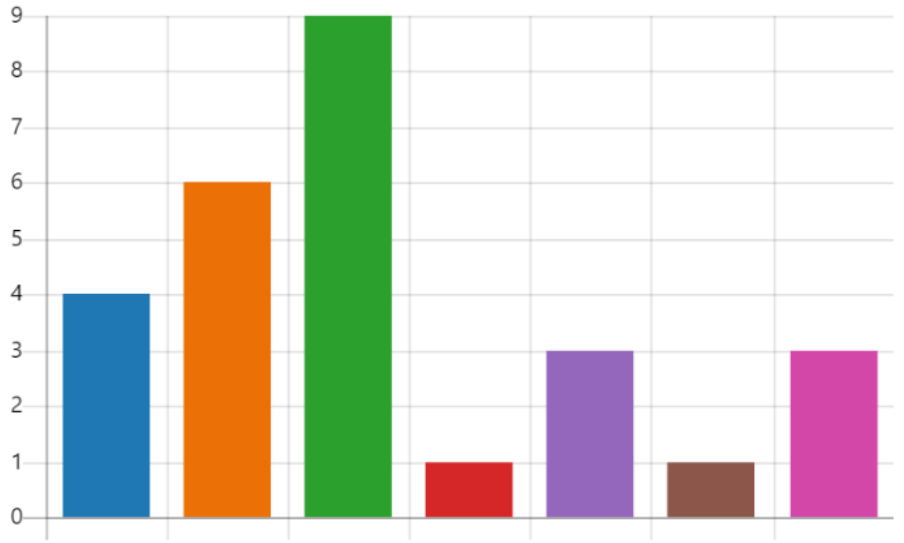
1. Does your health service use a decision algorithm to make decisions about **Formulary Applications** for high cost medicines?

Always	2
Often	2
Sometimes	7
Seldom	3
Never	5

Non-urgent IPU: decision making body

Where are **non-urgent** decisions for **Individual Patient Use Applications** for high cost medicines made? Select all that apply.

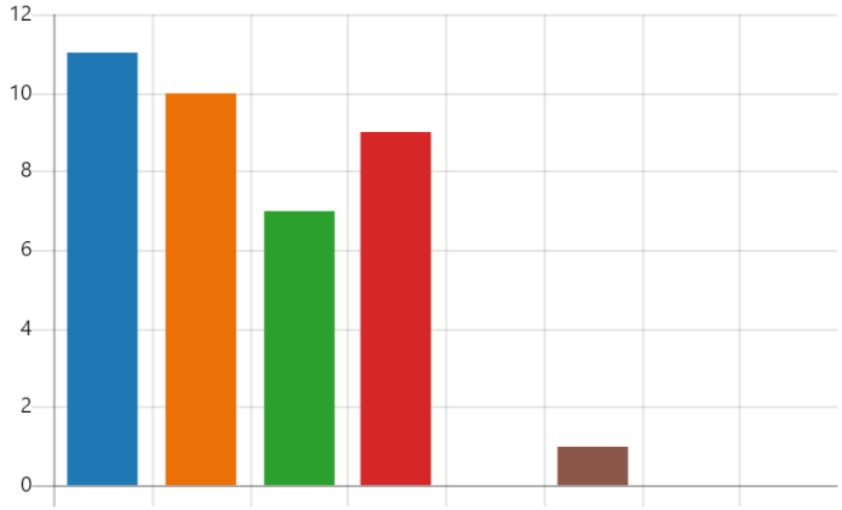
Group specifically to assess *all*...	4
Group specifically to assess In...	6
Medicines (Drug) and Therape...	9
High Cost Medicines Group/C...	1
Local Pharmacy Department d...	3
By an individual(s) within the ...	1
Other	3



Non-urgent IPUs: engage applicant (GP3)

. What support is provided to applicants when submitting a **non-urgent Individual Patient Use Application** for a high cost medicine?
(e.g. is the application screened and/or amended by someone prior to going to the committee/group for approval) Select all that apply.

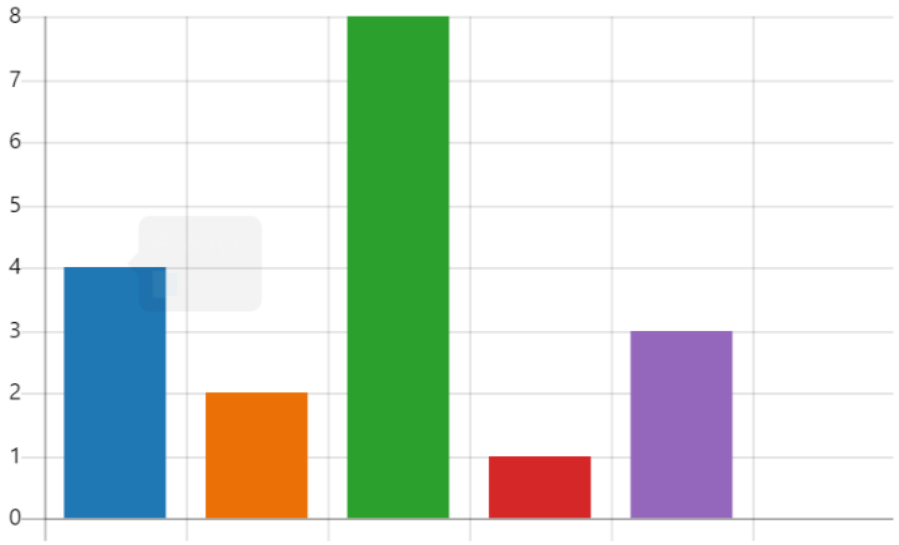
Director of Pharmacy (or dele...	11
Formulary pharmacist	10
Medical specialist/fellow	7
Head of medical unit submitti...	9
Electronic forcing functions in ...	0
Electronic forcing functions in ...	1
No support provided	0
Other	0



Non-urgent IPUs: algorithm (GP4)

Does your health service use a decision algorithm to make decisions about **non-urgent Individual Patient Use Applications** for high cost medicines?

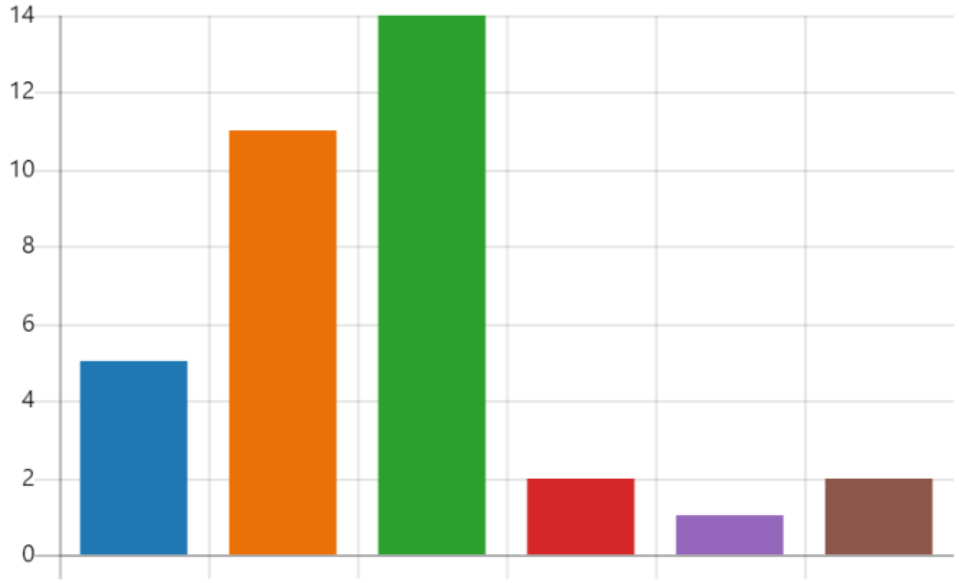
Always	4
Often	2
Sometimes	8
Seldom	1
Never	3
Other	0



Decision documentation (GP6)

In what format are decisions regarding high cost medicines kept?

Paper based applications kept...	5
Applications stored electronic...	11
Summary record of applicatio...	14
Dedicated electronic system u...	2
Dedicated electronic system u...	1
Other	2



Current state of play: straw poll

Has your health service incorporated the CATAG Guiding Principles for the governance of high cost medicines into practices at your health service?
Link to CATAG Guiding Principles: https://catag.org.au/resource/navigating-high-cost-medicines/

Fully incorporated/implement...	2
Partially incorporated/implem...	12
Somewhat, have reviewed but...	3
No, have not reviewed/imple...	1
Unsure, have not seen the Gui...	1