

information related to the criteria considered in decision-making for each medicine and its associated indication (i.e. a medicine-indication pair [MIP]). The criteria considered in decision-making included the comparator (therapy to which it was compared), type of economic analysis, accepted value, budget impact, financial cost of supply, cost of therapy per patient, access control (such as restrictions or prior authorization), and clinical need. Associations between types of MEA and the criteria were assessed using Chi Squared test.

RESULTS:

There were 87 MIPs, of which 56 had only financial MEAs and 31 had performance-based MEAs. Coverage with evidence development MEAs had very high incremental cost-effectiveness ratio (ICER)/quality adjusted life year (QALY) (74 percent > AUD 50,000 [USD 37,822]). Financial MEAs where performance measures were linked to reimbursement had lower ICER/QALY (13 percent > AUD 50,000 [USD 37,822]) but greater budget impact (33% > AUD 80million [USD 60.5million]) compared to simple financial MEAs. A statistically significant association (Cramer's V = 0.5, p < 0.001) was only found between performance-based MEAs and the cost of unsubsidized therapy per patient.

CONCLUSIONS:

The main influence on the choice of performance based MEA was the provision of access to clinically important medicines with a high treatment cost for patients.

OP18 A Patient And Caregiver-Designed Framework For Managed Access Programs

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INTRODUCTION:

Reimbursement decisions on orphan drugs carry significant uncertainty, and as the amount increases, so does the risk of making a wrong decision, where harms outweigh benefits. Consequently, patients often face limited access to orphan drugs. Managed access programs (MAPs) are a mechanism for managing risk while enabling access to potentially beneficial drugs.

Patients and their caregivers have expressed support for these programs and see patient input as critical to successful implementation. However, they have yet to be systematically involved in their design. The objective of this study was to explore what a framework for MAPs might look like when designed by patients and caregivers.

METHODS:

Building upon established relationships with the Canadian Organization for Rare Disorders, the project team collaborated with patients and caregivers using the principles of participatory action research. Data were collected at two workshops and analyzed using a thematic network approach.

RESULTS:

Patients and caregivers identified six aspects of an ideal MAP relating to accountability (program goals), governance (program-specific committee oversight; patient input; international collaboration), and evidence collection (outcome measures and stopping criteria; ongoing monitoring and registries). Additionally, patients and caregivers recognized that health care resources are finite and considered disease or drug eligibility criteria for deciding when to use a MAP (e.g. drugs treating diseases for which there are no other legitimate alternatives).

CONCLUSIONS:

A patient and caregiver-designed framework was created, which emphasized patient involvement and transparency. Further research is needed to examine the feasibility of this framework and roles for other stakeholders.

OP19 Are Compassionate Use Programmes Good Predictors of Clinical Benefit?

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INTRODUCTION:

In cases of high unmet clinical need, patients can access drugs prior to marketing authorization (MA) and Health Technology Assessment (HTA) through compassionate