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



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use programmes (CUP) or special access pathways (SAP). In theory, accelerated access is beneficial for patients with few therapeutic alternatives. In practice, it remains unclear if early access products actually deliver meaningful clinical benefit.

### METHODS:

Seventy-five drug-indication pairs were identified that have proceeded through a CUP or SAP in one or more countries including Canada, Australia, France, Sweden, England, and Scotland. Data was collected from regulatory and HTA websites on length of CUP or SAP, time prior to MA, time prior to HTA decision, time between MA and HTA decision, French Transparency Commission added clinical benefit (ASMR), and HTA decision. Cohen kappa scores were calculated in order to assess inter-agency agreement.

### RESULTS:

Across the 75 drug-indication pairs, average time between CUP and marketing authorization was 243 days, and average time between MA and HTA decision was 252 days. No products were deemed to be of major added clinical benefit (ASMR I), only 2.7 percent of products had important added clinical benefit (ASMR II), 26.7 percent of products had moderate added clinical benefit (ASMR III), 40.0 percent of products had minor added clinical benefit (ASMR IV), and 22.7 percent of products had no added clinical benefit (ASMR V). There is little inter-agency agreement in HTA recommendations for products that have proceeded through a CUP. The highest amount of agreement was seen between Canada and Scotland ($k = 0.24$).

### CONCLUSIONS:

Preliminary results suggest that CUP and SAP products accelerate access, but often only provide only moderate or minor improvements in clinical benefit. Further, there is very little agreement across HTA agencies on the value of these products.

## OP20 When Are Nationally Available Discounts Introduced In NICE Appraisals


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### INTRODUCTION:

Offering a nationally available discount has become common to increase the chance of being recommended by the National Institute of Health and Care Excellence (NICE). This study reviewed all NICE technology appraisals (TAs) since October 2007 to determine whether a national available discount was submitted, and explore when these discounts were introduced.

### METHODS:

All TAs between October 2007 and August 2017 were reviewed. The timing of the nationally available discount submission was allocated into one of four categories: initially submitted; initially submitted but changed; introduced after submission; or, other discount. An analysis was conducted to examine whether there was a temporal pattern in the introduction of nationally available discounts before or after January 2014, when the current Pharmaceutical Price Regulation Scheme (PPRS) came into effect.

### RESULTS:

Before 1 January 2014, a nationally available discount was only used in the minority of cases across recommended (22 percent of cases) and not recommended (19 percent) technologies. In the period since 1 January 2014, use of a nationally available discount increased overall, but to a greater degree in technologies ultimately receiving a positive recommendation from NICE (not recommended: 19 percent to 39 percent; recommended: 22 percent to 59 percent). In the period since 1 January 2014, the proportion of technologies with a positive recommendation where implicit price flexibility during the appraisal was revealed increased (from 20/186) to 40/182.

### CONCLUSIONS:

With the current PPRS, the majority of technologies have offered a nationally available discount, most commonly at the time of submission; however, there is increasing evidence of implicit price flexibility during the appraisal process to achieve a positive recommendation.

## OP23 Setting The Value Of New Technologies - A Survey


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