INTRODUCTION:

We examined relationships between measures of total knee arthroplasty (TKA) "appropriateness" constructs and surgeon TKA recommendations in people with knee osteoarthritis (OA). Although TKA is highly effective, fifteen to thirty percent of recipients report dissatisfaction and/or little or no symptom improvement. More appropriate selection of surgical candidates may improve both patient outcomes and healthcare resource use, but no validated appropriateness criteria exist currently in Canada.

METHODS:

Patients 30 years of age or older with knee OA referred for surgical consultation at two large joint arthroplasty centres in Alberta, Canada were invited to participate. Participants completed a standardized pre-consult questionnaire, which included the following sociodemographics and validated measures of appropriateness constructs for TKA: knee symptoms; non-surgical management; patient readiness for and expectations of TKA; and net patient benefit. Post-consultation, surgeons were asked to confirm knee OA and their recommendation. We used multivariable logistic regression to examine the relationship between measures of appropriateness constructs and receipt of surgeon TKA recommendation.

RESULTS:

Of 3,009 patients approached, 2,360 completed the questionnaire and 2,064 (sixty-nine percent) were eligible at surgical consultation (mean age 65.7 years, standard deviation 9.1; fifty-nine percent were women); 1,495 (seventy-two percent) were recommended for TKA. The likelihood of receiving a TKA recommendation was independently associated with: knee symptoms (odds ratio [OR] per unit increase in pain intensity, 1.19 (95% confidence interval [CI]: 1.11–1.27)); prior non-surgical OA management (OR for prior knee injection, 1.53 (95% CI: 1.21–1.94)); readiness for surgery (OR if definitely/probably willing to undergo TKA, 3.03 (95% CI: 1.99–4.59)); and TKA expectations (OR outcome "very important": ability to perform daily activities, 1.40 (95% CI: 1.04–1.88); straighten the knee/leg 1.42 (95% CI: 1.13–1.80); participate in exercise/sports 0.75 (95% CI: 0.58–0.98)).

CONCLUSIONS:

In our cohort of patients with confirmed knee OA who consulted a surgeon for TKA, appropriateness constructs were significantly associated with receipt of a TKA recommendation. Research is ongoing to evaluate the predictive validity of these measures for patient-reported outcomes associated with TKA.

OP163 European Network for Health Technology Assessment Joint Action 3 Relative Effectiveness Pilots: Pharma Company Experience

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INTRODUCTION:

More than 50 HTA agencies evaluate the clinical value of medicines in Europe, resulting in duplication of work for HTA agencies and manufacturers, and lengthy and variable time to reimbursement for patients across Europe. A consistent, single European relative clinical scientific benefit assessment of medicines could become a key element in ensuring patients get equitable and timely access across Europe. The European Network for Health Technology Assessment (EUnetHTA) is responsible under Joint Action 3 (JA3, 2016–2020) to pilot more than 30 Relative Effectiveness Assessments (REAs) of medicines. The first EUnetHTA JA3 REA pilots are now being completed and Roche, with its participation in the REA pilot for alectinib, has gathered relevant experience.

METHODS:

The goal of this analysis is to summarize and reflect upon the experience with one of the first EUnetHTA REA assessments in JA3. The authors also propose potential process improvements.

RESULTS:

The experience with the alectenib REA shows that EUnetHTA processes have improved compared to JA2. The timing of the assessment has been aligned with the EU regulatory marketing authorization process by shortening the duration of the scoping phase. More EUnetHTA members than in JA2 seem to be committed to use the reports in national HTA, pricing and reimbursement processes. At the same time, the REA

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pilots have identified other areas that could benefit from further refinement, for example the active engagement of patient group representatives and clinical experts, rules and principles related to the handling of confidential information.

CONCLUSIONS:

Based on the limited number of REA pilots for medicines it is too early to draw final conclusions on the state of EU-level collaboration. But first signals indicate a positive development compared to REA pilots conducted in JA2. Interim evaluations are recommended to assess progress, and capture learnings for future pilots.

OP164 Hospital Budget Impact Of High-Cost Drugs: The Case Of Nusinersen

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INTRODUCTION:

Nusinersen is an orphan drug for spinal muscular atrophy (SMA) recently approved for marketing. Its high cost, striking but limited evidence of efficacy, and strong demand by media and patient organizations have generated a health policy conflict. We analyze the flaws of available evidence on nusinersen and its budget impact at a pediatric hospital, and report a collaborative strategy for drug procurement and financing.

METHODS:

Nusinersen is the highest-cost drug assessed by our hospital-based health technology assessment (HB-HTA) program so far. At the time of our assessment, only interim-analysis data of the pivotal randomized trial submitted to Federal Drug Administration (FDA) for approval and the European Medicines Agency (EMA) report containing unpublished final results were available. These secondary sources and other published phase II results were appraised. As a referral hospital, we concentrate most of the 300 SMA patients in our country. Hospital budget impact estimation included drug and hospitalization costs for the first and following years. The HTA report was submitted to the Ministry of Health to address this financing issue.

RESULTS:

The available evidence of efficacy raised serious methodological and clinical uncertainties. First-year treatment cost per patient was estimated in ARS 13,008,688 (USD 752,000, 10 percent of pharmacy annual drug budget). Hospital budget impact (70 eligible patients) was ARS 910,608,160 (USD 52,000,000; 18 percent of total annual hospital budget). Our recommendation was to contact central level authorities to resolve both drug financing and patient access by negotiating a shared-risk approach for an expanded access program, allowing further data collection for reassessment after 12 months. This, in turn, fostered mutual collaboration and consensus within the health system where several lawsuits were demanding drug coverage. Negotiation with the industry was initiated by the Ministry.

CONCLUSIONS:

This case is a clear example of forthcoming ultra-high-cost drugs unaffordable by hospital budgets. Their acquisition opportunity cost is a health policy matter requiring to display collaborative coping strategies with Ministries and other stakeholders including industry.

OP166 How Responsive Is Industry To Value Based Procurement?

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INTRODUCTION:

The European Union (EU) directive (2014/24) on public procurement strives to stimulate innovation and seeks for methodologies to implement a quality/cost based approach to search for the most economically advantageous tendering (MEAT). MedTech Europe launched the MEAT value-based-procurement (VBP) framework and tool which considers product's value from different perspectives/dimensions. Results from the first EU pilot, testing the feasibility to use the MEAT framework at a university hospital, are presented.

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