specific subgroup, and place in treatment. Stage of disease was the only element where data was either not generated for the IHSI database's estimated indication, not aligned with the HTD's proposed indication, or reported in an incorrect field.
Conclusions: There is a high degree of alignment between an HTD-proposed indication and the IHSI-estimated indication. The processes for generating an estimated indication will involve both NLP-generation and human co-curation. The current (curator-selected) elements are being used to train the NLP engine. Thereafter, the engine will process clinical trial data to surface tags for human selection to generate the structured inputs.

OD27 Estimating The Causal Effect Of Adaptive Treatment Strategies Using Longitudinal Observational Data

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Introduction: Real-world data can help inform policymaking in health care by facilitating the evaluation of realistic treatment protocols. To generate robust evidence, analysts must address time-dependent confounders—variables influenced by past treatment decisions and affecting future treatment. Double-robust methods can help in eliminating bias by modeling both the treatment and the outcome mechanisms, using machine learning to improve model specification.
Methods: Longitudinal targeted minimum loss-based estimation (LTMLE) is a double-robust method that handles time-varying confounding, currently with only a few applications on real-world data. We demonstrate the use of LTMLE to evaluate realistic treatment protocols by applying it on longitudinal registry data to compare various treatment protocols that involve the use of erythropoiesis-stimulating agents (ESA) for myelodysplastic syndromes patients. We define dynamic regimes that trigger initiating ESA when relevant criteria (e.g., low hemoglobin levels) are met and require continuing/stopping ESA based on the response to treatment. We estimate the effect of these protocols on survival and EuroQol 5-dimension questionnaire (EQ-5D) scores.
Results: We study static treatment regimes where we compare patients always on treatment with patients always not on treatment, and we find the average effects of always administering ESA versus never administering it are positive but not significant on patients' EQ-5D scores or on survival probabilities across all treatment time periods. We also study dynamic treatment regimes where decisions to initiate and continue/discontinue treatment over time depend on changing patient characteristics and responses to treatment. We find that patients following dynamic treatment regimes are predicted to score higher in EQ-5D and have longer survival probabilities than patients under static treatment regimes.
Conclusions: The paper provides a tutorial and case study demonstration of the LTMLE model that can evaluate realistic treatment protocols using longitudinal observational data. It accounts for time-varying confounding in estimating treatment effects and can incorporate machine learning in improving accuracy of outcome prediction. The model has been applied in the setting of long follow-up times and gradually reduced sample size.

OD28 Towards Implementing New Payment Models For The Reimbursement Of High-Cost, Curative Therapies: Insights From Semi-Structured Interviews

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Introduction: In response to the intricate challenges posed by high-cost, one-shot curative therapies, this study explores what hinders the wide implementation of innovative payment schemes across Europe. Drawing insights from the Belgian social healthcare system, this study focused on defining the necessary and sufficient conditions for implementing outcome-based spread payments in the context of market access to advanced medicinal products
Methods: Semi-structured interviews (n=33) were conducted with physicians (n=2), hospital pharmacists (n=4), hospital managers (n=2), patient representatives (n=3), industry representatives (n=5), Belgian policymakers (n=6), sickness fund representatives (n=4), legislative experts (n=2), and accounting experts (n=5) to elicit opinions and insights on stakeholders' responsibilities and roles, and identify the necessary and sufficient conditions to establish outcome-based spread payments for the reimbursement of innovative therapies. The interviews took place between July 2020 and October 2020. The framework method analysis was performed using NVivo software (version 20.4.1.851). Statements were allocated into six main topics: payment structure, spread payments, outcome-based agreements, governance, transparency, and regulation.
Results: Interviewees across stakeholder groups endorsed the idea of implementing outcome-based spread payments. However, opinions varied on practical and legal feasibility, especially regarding long-term follow-up for patients, data collection burden on physicians, and implications on the financing flow of health technology developers, hospitals, and the government. Concerns were also raised regarding the potential need for new governance structures, enhanced transparency on agreements and pricing mechanisms, as well as defining data requirements to address uncertainties often seen with this type of therapy. All interviewees emphasized the importance of increasing stakeholders' understanding of these agreements to foster broader acceptance and successful implementation.
Conclusions: The effective implementation of outcome-based spread payments falls behind because consensus on how this reimbursement

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method can be a sustainable solution is missing. Leveraging the concepts of necessary and sufficient conditions from organizational research, this study provides guidance on resolving challenges and defines stakeholders' roles for successfully implementing this reimbursement approach.

OD29 From Listening To Lift Off: Developing A Three-Year Public Involvement And Engagement Strategy For NICE

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Introduction: Involving patients in the health technology assessment (HTA) lifecycle is a core principle at the National Institute for Health and Care Excellence (NICE). To achieve this, NICE has adopted a mixed approach to patient and public involvement and engagement (PPIE) spanning the entire appraisal process. To ensure the PPIE approach enables meaningful involvement, NICE engaged with stakeholders to review its effectiveness and identify areas for improvement.
Methods: In 2023, an independent consultant reviewed NICE's PPIE approach and engaged with NICE staff and external stakeholders from patient organizations, individual patient contributors, and engagement leads at national health and social care organizations. The engagement included interviews with NICE staff (n=19) and external stakeholders (n=13), and an online survey that received 83 responses from patient organizations and patient contributors. Using this feedback, NICE's patient and public involvement program conducted four focus groups to develop a framework of improved methods and processes for PPIE with NICE staff, patient organizations, and patient contributors.
Results: The engagement identified many positives in NICE's approach to PPIE, including:

- lay members sitting on each HTA committee as equal members
- patient organizations providing written evidence to HTA committees
- patient experts providing written and verbal testimony to HTA committees
- support provided by NICE.

The engagement also identified areas where PPIE could have a greater impact, including:

- improved methods for collecting patient evidence and insight
- strengthening the role of lay members
- collating and reusing previously collected patient evidence
- taking a proportionate approach to involving small organizations
- allocating staff resources to focus on impactful PPIE practices.

Conclusions: NICE has developed a draft framework for an improved approach to increase the impact of PPIE in HTA decision-making. In 2024, NICE will publicly consult with NICE staff and external stakeholders to review the framework, agree the strategic aims, and develop metrics for measuring success. Following this consultation, the findings and NICE's updated approach to PPIE will be presented.

OD30 Clinician-Driven Health Technology Assessment: National Cancer Medicines Review For Off-Label Uses And On-Label Off-Patent Uses In NHSScotland

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Introduction: Publicly funded cancer services face significant financial and capacity challenges. It is estimated that 40 percent of medicines used to treat cancer are outside their marketing authorization or "off-label." These uses are usually outside the remit of health technology assessment (HTA) groups. Accessing emerging off-label uses is mostly through individual patient requests, which are resource intensive, delay patient treatment, and produce inequity.
Methods: A program providing national HTA review of off-label and off-patent cancer medicine uses has been established by Healthcare Improvement Scotland. Processes include horizon scanning, support for proposing clinicians, and engaging patient groups. Relevant published and unpublished clinical and cost-effectiveness information—identified through systematic literature searches, engagement with pharmaceutical companies, academic and health service data groups—supports independent appraisal and decision-making. Where cost-effectiveness information is unavailable, a value-judgment framework, including magnitude of clinical benefit, uncaptured benefits, and budget and service impact, is utilized to standardize review. The decision-making Council includes public partners, and advice is shared across NHSScotland.
Results: From July 2022 to October 2023, the program has published advice on nine proposals—eight off-label uses and one on-label off-patent use. Health economic models from a pharmaceutical company and an academic group supported decision-making on two proposals, value-judgment frameworks for two proposals, and real-world evidence for one proposal. Eight proposals were supported, and one was not supported. Each supported proposal slowed cancer, prolonged life, or reduced toxicity compared to standard treatment options. Four were cost-saving and three had a low medicines budget

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