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# Oral Presentations

## OP117 Digital Real-World Evidence In Times Of General Data Protection Regulation

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**Introduction.** Real-world evidence (RWE) is a useful supplement to a product's evidence base especially for medical devices, which are often unsuitable for randomized controlled trials. Generally, RWE is analyzed retrospectively (for example, healthcare records), which lack granularity for health-economic analysis. Prospective collection of RWE in hospitals can promote device-specific endpoint assessment. The advent of the General Data Protection Regulation (GDPR) requires a privacy-by-design approach. This work describes a workflow for a GDPR-compliant device-specific RWE collection as part of quality improvement initiatives (QII).

**Methods.** A literature review identifies relevant clinical and quality markers as endpoints to the investigated technology. A panel of experts grade these endpoints on their clinical significance, privacy sensitivity, analytic value, and feasibility for collection. Endpoints meeting a predefined cut-off are considered quality markers for the QII. Finally, an RWE data collection app is designed to collect the quality markers using either longitudinal, pseudonymized data or single time-point anonymized data to ensure data protection by design.

**Results.** Using this approach relevant clinical markers were identified in a GDPR-compliant manner. The data collection app design ensured that patient data were protected, while maintaining minimum requirements on patient information and consent. The pilot QII collected data on over 5,000 procedures, which represents the largest single data set available for the tested technology. Due to its prospective nature this programme was the first to collect patient outcomes in sufficient quantity for analysis, while previous studies only recorded adverse events.

**Conclusions.** GDPR and RWE can co-exist in harmony. A design approach, which has data protection in mind from the start can combine high quality RWE collection of efficacy and safety data with maximum patient privacy.

## OP123 The Use Of Surrogate Outcomes In National Institute For Health And Care Excellence (NICE) Highly Specialised Technology Evaluations: A Review Of Published Guidance

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**Introduction.** The use of surrogate outcomes in health technology assessment (HTA) is increasing and methods for validating surrogate relationships have been published. However, these may not be fully applicable to ultra-rare diseases due to challenges such as scarcity of evidence and heterogenous populations. This study reviews and summarizes the use of surrogate outcomes and committee's considerations in the evaluations within the National Institute for Health and Care Excellence's (NICE) Highly Specialised Technology (HST) programme, which was established in 2013 in response to the challenges associated with the assessment of ultra-rare diseases.

**Methods.** All HST evaluation documents published before November 2020 were reviewed. Data extracted included surrogate outcomes used, rationales, the committee's considerations on the validity and generalizability of the surrogate relationships, related uncertainties, and other factors considered in decision-making.

**Results.** Seven out of the eighteen published HST topics used surrogate outcomes. The rationale for most of the surrogate relationships focused on biological plausibility. Common concerns raised by the committee included the generalizability of the surrogate relationship to the condition of interest, the lack of validation, and inability to prove or quantify the magnitude of benefits associated with the surrogate relationships. In some topics, other aspects of the evidence and clinical/patient expert's opinions were also considered by the committee.



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**Conclusions.** The use of surrogate outcomes is common in NICE HST evaluations and the challenges in supporting surrogate relationships with more than biological plausibility are recognized. However, our review indicates that, the committee considers more than just biological plausibility and will take into account other related factors.

## OP128 Improving Literature Searching For Evidence On Health Apps: The National Institute For Health And Care Excellence (NICE) MEDLINE And Embase (Ovid) Health Apps Search Filters

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**Introduction.** Literature searching for evidence on apps in bibliographic databases is challenging because they are often described with inconsistent terminology. Information Specialists from the United Kingdom's National Institute for Health and Care Excellence (NICE) have developed validated search filters for retrieving evidence about apps from MEDLINE and Embase (Ovid) reliably.

**Methods.** Medical informatics journals were hand-searched to create a 'gold standard' set of app references. The gold standard set was divided into two sets. The development set provided the search terms for the filters. The filters were validated by calculating their recall against the validation set. Target recall was >90%.

A case study was then conducted to compare the number-needed-to-read (NNR) of the filters with previous non-validated MEDLINE and Embase app search strategies used for the 'MIB214 myCOPD app' NICE topic. NNR is the number of references screened to find each relevant reference.

**Results.** The MEDLINE and Embase filters achieved 98.6 percent and 98.5 percent recall against the validation set, respectively. In the case study they achieved 100 percent recall, reducing NNR from 348 to 147 in MEDLINE and from 456 to 271 in Embase.

**Conclusions.** The novel NICE health apps search filters retrieve evidence on apps from MEDLINE and Embase effectively and more efficiently than previous non-validated search strategies used at NICE.

## OP129 The Use Of A Text-Mining Screening Tool For Systematic Review Of Treatments For Relapsed/Refractory Diffuse Large B-Cell Lymphoma

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**Introduction.** Human screening of title and abstracts in a systematic literature review (SLR) is labor intensive and time-consuming. In many instances, thousands of citations may be retrieved; the vast majority excluded upon screening. Text-mining semi-automates and accelerates screening by identifying patterns in relevant and irrelevant citations, as labelled by the screener. One such text-mining tool, Abstrackr, uses an algorithm within an active-learning framework to predict the likelihood of citations being relevant. The objective of this study was to assesses the performance of Abstrackr for title and abstract screening in an SLR of treatments for relapsed/refractory diffuse large B-cell lymphoma.

**Methods.** Citations identified from searches of electronic databases were imported to Abstrackr. An investigator-selected database of terms indicating relevance of title and abstract to the research question were uploaded. These terms were partly informed by the SLR inclusion/exclusion criteria. Citations deemed most relevant by Abstrackr were screened first (screening prioritization). Screening was carried out until a maximum prediction score of 0.4 or less, based on previous experience in the literature, was reached. Remaining citations were deemed unlikely to be relevant and did not undergo screening (screening truncation). Separately, a single-human screener screened all citations using Covidence.

**Results.** A total of 7,723 citations and 154 initial terms were uploaded to Abstrackr. Of these citations, 2,572 (33 percent) were screened before a prediction score of 0.39 was reached. Compared to single-human screening (conducted on all citations), the workload saving associated with Abstrackr was 5 days. A total of 451 (6 percent) citations proceeded to full-text screening; ten (0.1 percent) were included in the final evidence base. No citations predicted to be irrelevant by Abstrackr were included in the final evidence base.

**Conclusions.** Text-mining tools such as Abstrackr have the potential to reduce workload associated with title and abstract screening, without missing relevant citations.

## OP130 Economic Evaluation Of High-Cost Drugs For Relapsing-Remitting Multiple Sclerosis In Thailand

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**Introduction.** Drugs for relapsing-remitting multiple sclerosis (RRMS) are costly and not included in the National List of Essential Medicines of Thailand yet. This study aims to conduct an economic evaluation of high-cost drugs for RRMS.

**Methods.** The Markov model was used to estimate lifetime costs and quality-adjusted life years (QALYs) gained. The treatment options include Interferon beta-1a (IFN) and Teriflunomide (TERI) (first-line), Fingolimod (FIN) and Natalizumab (NATA) (second-line), and Alemtuzumab (ALEM) (third-line) compared with usual care. The effectiveness of drugs was retrieved by network meta-analysis. The probability of health state transition was obtained from primary data. Treatment-related costs were derived from the national database. Other costs and utilities were obtained from the study in Thai RRMS patients.

**Results.** The lowest lifetime costs option was usual care (THB2 million) (USD65,808), while the highest QALY gained option

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