International Journal of Technology Assessment in Health Care

cambridge.org/thc

Oral Presentations

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

Rhodri Saunders, Rafael Torrejon Torres and Maximilian Blüher (mb@coreva-scientific.com)

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

Yelan Guo (yelan.guo@nice.org.uk), Caroline Bregman and Nicole Elliott

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.

CAMBRIDGE UNIVERSITY PRESS

https://doi.org/10.1017/S0266462321000660 Published online by Cambridge University Press