PP121 Assessment Of Heart Rate Characteristics Analysis

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Introduction: The heart rate characteristics (HRC) analysis is a technique for predicting and monitoring neonatal infectious diseases in infants with a risk for infectious disease, such as sepsis, using the HRC index based on the electrocardiogram (ECG) data transmitted from a HeRO® device connected to an ECG monitor.
Methods: The assessments were performed via a systematic review. Searching five databases (KoreaMed, Ovid-MEDLINE, Ovid-EMBASE, and Cochrane) yielded 284 related studies. Two reviewers independently assessed the quality of these studies, using the Scottish Intercollegiate Guidelines Network checklist and the assessment results were described based on the results of the quality appraisal and level of evidence.
Results: The safety and effectiveness of the technique were assessed based on eight non-Korean studies (one randomized controlled trial (RCT), five cohort studies, one case-control study, one case series). The four studies that reported area under the curve (AUC) values, the timing of the analysis and AUC values (0.67– 0.75) were inconsistent among the studies. The elevation of the HRC index prior to the diagnosis of an infectious disease was not observed for meningitis or for multiple infectious diseases. In addition, although the group with additional monitoring using the technique had a significantly lower mortality rate, whether the HRC index had a direct impact on the infection prevention activities and the possibility of bias arising from the researchers' precautionary treatment based on the clinical symptoms and other test results as well as the actual time of disease onset are unknown.
Conclusions: Therefore, the committee presented the following recommendations based on the available evidence and expert opinion. HRC analysis is a safe technique for predicting and monitoring neonatal infectious diseases in infants with a risk for infectious disease, such as sepsis, using the HRC index based on the ECG data transmitted from a HeRO® device connected to an ECG monitor; however, further research is needed to determine its effectiveness (Level of evidence C, Technology category II-a).

PP122 The Feasibility Assessment For Domestic Introduction Of Newborn Pulse Oximetry Screening For Critical Congenital Heart Disease

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Introduction: Critical congenital heart disease (CCHD) refers to a group of heart defects that cause serious, life-threatening symptoms in the neonatal period and requires timely surgical or catheter interventions. We reviewed evidence for incorporating a mandatory neonatal CCHD screening test as a national public health project for all neonates born in Korea by analyzing the validity and cost-effectiveness of neonatal CCHD screening using pulse oximetry in Korea.
Methods: We performed a rapid literature review to establish models for the diagnostic accuracy and economic evaluation of pulse oximetry. Also, we analyzed the prevalence, mortality, and medical expenditure for different types of CCHD using the national health insurance (NHI) data. We analyzed the cost-effectiveness of pulse oximetry by comparing the group of neonates who received a combination of a physical examination and pulse oximetry, and group of neonates who only received a physical examination. For the cost-effectiveness analysis for the CCHD screening test in this study, we used a duration of one year, diagnostic accuracy as the clinical endpoint, and Life Year Gain (LYG) as the effectiveness indicator.
Results: We used recent systematic review he pooled sensitivity can be enhanced from 76.5 percent (pulse oximetry alone) to 92 percent (combined with physical examination). We used a total of 2,334 neonates with CCHD data for the economic model. Our analysis revealed that adding pulse oximetry to the routine neonatal physical examination leads to 2.34 of LYG and a cost difference of USD1,080,602, showing a ICER of KRW610,063,240 (USD461,857)/LYG.
Conclusions: Considering the benefit of LYG and cost of reducing the complications and after effects of newborns with CCHD who survived early diagnosis, it is considered to be worthwhile in Korea for a mandatory screening test.

PP124 Patisiran For Patients With Hereditary Transthyretin Amyloidosis (hATTR) With Stage 2 Polyneuropathy Or Who Have An Inadequate Response To Tafamidis

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Introduction: Hereditary Transthyretin amyloidosis (hATTR) is a rare autosomal dominant, multisystemic, progressive, and potentially fatal genetic disease. Currently, the only drug made available in the Brazilian National Health System to treat hATTR is tafamidis meglumine, indicated for symptomatic adult patients in early stage (stage 1) and not undergoing liver transplantation for amyloidosis associated with hATTR.

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Methods: A systematic review was conducted in the databases MEDLINE via Pubmed, Embase, The Cochrane Library, and LILACS addressing the question "Is patisiran treatment effective and safe for patients diagnosed with ATTRh amyloidosis with stage 2 polyneuropathy or who have an inadequate response to tafamidis?"
Results: The 13 studies included in the review demonstrate the efficacy of patisiran in reducing the neuropathic progression of the disease, as evidenced by decreased mNIS+7 scale scores following 18-month use of the drug. Improvements in the quality of life of patients taking patisiran have been reported, as measured by reduced scores on the Norfolk-QoL-DN scale. Patisiran has also been shown to be effective in reducing NT-proBNP, a marker related to cardiac stress. Improvements in the nutritional status of patients taking patisiran were demonstrated by increasing modified body mass index (BMI). Good tolerability of patisiran was observed by patients using it. Most adverse events were classified as mild or moderate. The studies indicated that the occurrence of deaths is similar between the patisiran and placebo groups. Most deaths were related to cardiac events and were not associated with the use of patisiran.
Conclusions: The use of patisiran in patients with hATTR demonstrated efficacy in reducing the neuropathic progression of the disease, evidenced by decreased mNIS+7 scale scores, improvements in quality of life as measured by reduced Norfolk-QoL-D scale scores, and reduced NT-proBNP. The drug patisiran was well tolerated, with most adverse events rated as mild and moderate.

PP125 Why Understanding The Burden Of The Population Is Fundamental

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Introduction: Despite intense efforts in development of new treatments over the last two decades, symptomatic treatments remain the only option for the vast majority patients diagnosed with dementia due to Alzheimer's disease (AD). There remains a significant unmet need for disease modifying therapies (DMTs) to slow or stop AD progression. DMTs in development are targeting early stages of AD (pre-clinical, mild cognitive impairment and mild dementia stages), thereby creating an entirely new treatment paradigm for patients, clinicians, and payers. A key challenge will be in identifying the appropriate patient for treatment in a very heterogenous population. We have performed a literature review to better understand and define the AD population, with a view to enabling more targeted treatment in future.
Methods: Embase, MEDLINE and the Cochrane Library were searched to identify publications between 2010-2021 on observational studies reporting evidence on prevalence and subgroup identification, including clinical feasibility of identification. The search was restricted to English language.
Results: We identified 45 studies, mostly from Europe, USA and Asia. Populations were primarily grouped based on generic demographic factors (e.g., age, sex, gender), AD staging, comorbidities or biomarkers. Prevalence data was available for six subpopulations: pre-dementia stage, mild dementia, age, Apolipoprotein E (APOE) genotype, comorbid obesity and hypertension. Across these, data on prevalence were heterogenous depending on study design and country of origin, and ranging between 66 million to 102 million for people with mild AD dementia, or as another example, ranging between 46 million to 92 million for APOE genotype carriers worldwide.
Conclusions: The heterogeneity and the uncertainty in prevalence of the AD population represent big challenges to clinicians and payers. Future discussions on target patient identification for new treatments should be aligned and integrated with current clinical practice e.g. leveraging validated biomarkers as diagnostic tools. Additional research on an integrated approach to identify patients who would benefit the most from DMTs will be needed.

PP127 Early Health Technology Assessment (HTA) Of Medical Technologies To Inform Subsidy Decision-making In Singapore

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Introduction: Medical technologies are evolving rapidly, with many new and expensive technologies entering the market constantly, challenging the sustainability of the public healthcare system. Early health technology assessment (HTA) to inform subsidy decision for innovative medical technologies, before they diffuse into the public healthcare system, may drive appropriate early adoption or curtail inappropriate use. This abstract describes the Agency for Care Effectiveness (ACE)'s experience in conducting early HTAs and key challenges faced.
Methods: During ACE's 2021 topic prioritization exercise, ACE took a proactive approach by considering medical technologies identified from horizon scanning (HS) for subsidy evaluation. Two topics were shortlisted from HS. Standard HTA evaluation framework and local clinician consultation were used to define the evaluation scope and clinical pathways. Literature search and appraisal were conducted for safety, effectiveness, and economic evidence. Budget impact estimations and organizational feasibility assessment were additional domains considered for subsidy decision-making by the Ministry of Health Medical Technology Advisory Committee (MTAC).
Results: MTAC did not recommend subsidy for the two technologies due to weak evidence base, largely due to a lack of comparative evidence, small samples, short-term follow-ups, or heterogeneity of population. Additional considerations included potentially high budget impact or organizational feasibility issues such as substantial capital and maintenance cost and infrastructure reconfiguration required. During the evaluation, key challenges of assessing such technologies in their early diffusion within the healthcare system were: (i) differing clinical opinions on whether the technology meets an unmet need; (ii) uncertain place in the clinical management algorithm for the relevant indications; (iii) sparse and weak evidence;

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