International Journal of Technology Assessment in Health Care

www.cambridge.org/thc

Oral Presentations

OP01 Impact Of Cost Heterogeneity On Assessing The Value Of Gene Therapies

Antal Zemplenyi (antal.zemplenyi@cuanschutz.edu), Jim Leonard, Michael DiStefano, Kelly Anderson, Garth Wright, Nick Mendola, Kavita Nair and Robert Mcqueen

Introduction: High-cost gene therapies strain the sustainability of healthcare budgets. Despite the potential long-term savings promised by certain gene therapies, realizing these savings faces challenges due to uncertainties regarding the treatment's durability and a lesser-discussed factor: the true potential for cost offset. Our study aims to assess the cost-offset uncertainty for US Medicaid regarding recently approved gene therapies in hemophilia A and B.
Methods: The analysis used 2018 to 2022 Colorado Department of Health Care Policy & Financing data to determine direct costs of standard of care (factor replacement therapy or emicizumab). Cost-simulation models over five- and ten-year time horizons estimated Colorado Medicaid costs if patients switched to gene therapy (valoctocogene roxaparvovec or etranacogene dezaparvovec) versus maintaining standard of care. Patients were included if aged 18 and over with ICD-10-CM codes D66 (hemophilia A) and D67 (hemophilia B). In the base case, severe hemophilia A was defined as requiring greater than or equal to six yearly factor VIII or emicizumab claims and moderate/severe hemophilia B requiring greater than or equal to four factor IX replacement therapy claims annually.
Results: Annual standard-of-care costs were USD426,000 (SD USD353,000) for hemophilia A and USD546,000 (SD USD542,000) for hemophilia B. Valoctocogene roxaparvovec (hemophilia A) had incremental costs of USD880,000 at five years and −USD481,000 at 10 years. Sensitivity analysis revealed a 23 percent chance of break-even within five years and 48 percent within 10 years. Etranacogene dezaparvovec (hemophilia B) showed incremental costs of USD429,000 at five years and −USD2,490,000 at 10 years. Simulation indicated a 32 percent chance of break-even within five years and 59 percent within 10 years. Varying eligibility (≥4 to ≥15 standard-of-care claims) notably affected break-even; for example, valoctocogene roxaparvovec: 40 percent to 77 percent chance of break-even in 10 years.
Conclusions: Our study highlights significant cost variation in the standard of care of patients eligible for gene therapies, adding to the uncertainty surrounding cost estimation and highlighting the importance of addressing this factor in risk-sharing agreements. The impact of varying eligibility criteria on cost offsets emphasizes the importance of carefully defining eligibility when using real-world data in the context of health technology assessment.

OP02 Osseointegrated Prosthetic Implants (Direct Skeletal Attachment) For People With Lower-Limb Amputation: A Hospital-Based Health Technology Assessment

Elena Galfrascoli (elena.galfrascoli@gmail.com), Dario Concetto Pistritto, Maristella Ghiringhelli, Martina Sterpetti, Michele Bertoni, Silvia Bozzi, Federica Asperti and Raffaella Cavi

Introduction: Patients with amputation need a safe and comfortable connection with the prothesis. Traditional sockets may lead to skin tearing, pain, and limitation of movement. Osseointegrated protheses connected to residual bone may have a positive impact on patients' quality of life. Our research question: Are there economic and organizational benefits from the introduction of a direct skeletal attachment (DSA) procedure, as well as clinical benefits?

Methods: Firstly, a systematic literature review was carried out on PubMed and Scopus, and the Critical Appraisal Skills Programme (CASP) tool was applied to assess the evidence's quality. A health technology assessment (HTA) evaluation was conducted (EUnetHTA, 2016), adopting the perspective of a public hospital in Italy, comparing two scenarios—traditional and innovative—related to traditional protheses and the DSA approach. Seven experts (orthopedic surgery, physical medicine, and rehabilitation and physiotherapy) were involved for the administration of a qualitative questionnaire. For the economic evaluation, a cost-effectiveness analysis and budget impact analysis were defined. Finally, a multiple-criteria decision analysis was performed.
Results: The literature search yielded 314 citations published until December 2022: eight were eligible for analysis. Three were the system analysed: 'OPRA', 'ISP' and 'OPL'. The efficacy of the systems is linked to a better distribution of bone stress: an increase in bone mineral density was recorded near the implants (respectively 28%, 27%, and 18% – after 60 months). The safety of DSA depends on the design and integrity of the connection between tissues and implant. The impact on the budget is an increase of 27 percent in costs for each patient treated. Concerning the social and ethical implications, DSA results in the preferable approach (1.48 vs 0.34), as it can limit social costs (0.29 vs −0.29).
Conclusions: The comparative evaluation was carried out using a scoring method: the main advantages related to innovative prostheses are based on effectiveness and safety, as well as social impact and organizational impact, especially due to the ability of the prostheses to reduce the risk of adverse events and long rehabilitation, with important clinical benefits and organizational savings for the hospital management.

OP03 Regionalization And Patient-Centered Care: A Rapid-Realist Review And Implications For Health Technology Assessment

Joan Quigley (j.quigley.2@research.gla.ac.uk), Catherine O'Donnell and Neil Hawkins

Introduction: Healthcare regionalization is the movement of responsibility of health care towards a regional body. It has been introduced in many countries and exists in many forms across high-, middle- and low-income countries. Supporters of regionalization purport that a more actively managed system, with better coordination and integration, could lead to improved quality and patient-centered care. However, evidence for this is unclear.
Methods: Systematic searches combining terms for regionalization and patient-centered care in MEDLINE identified 5,765 titles for review. Three levels of screening were conducted by two independent reviewers: title only, abstract and title, and full-paper review. Rapid-realist synthesis methodology was used to gather a deeper understanding of the relationship between healthcare regionalization and patient-centered care, seeking to identify potential mechanisms and the context in which these operate. We also sought to determine whether novel methodologies such as this can be used by health technology assessment (HTA) bodies in an efficient manner that produces results directly applicable to decision-makers.
Results: Studies from high-income countries, including Canada, New Zealand, Australia, and Italy, were included. The realist synthesis identified mechanisms by which whole healthcare-system regionalization can help or hinder the rollout of patient-centered care. Mechanisms were classified in relation to specific dimensions of patient-centered care including access and "patient as person." Facilitators to the use of rapid-realist review in health policy include similarity of screening, searching, and extraction to traditional systematic review. Barriers include the scope of the literature considered relevant, length of time to familiarize with the method, and presentation of the findings in an accessible way for policymakers.
Conclusions: This is the first realist synthesis of the relationship between whole healthcare-system regionalization and patient-centered care. Regionalization may help or hinder achievement of patient-centered care. Policymakers should note barriers to, and facilitators of, patient-centered care in the context of large-scale health system reform. Rapid-realist review has applications for HTA, particularly in the exploration of non-standard interventions.

OP04 The Modernized Cardiff Model: Multifaceted Modeling In The Era Of Cardiovascular-Kidney-Metabolic Syndrome

Philip McEwan (phil.mcewan@heor.co.uk), Volker Foos, Jieling Chen, Marc Evans, Geraint Roberts, Robert Jenkins and Andrew Gibson

Introduction: In the era of cardiovascular-kidney-metabolic syndrome, thorough evaluation of medicines with multiple treatment effects/indications demands a multifaceted modeling philosophy, despite the requirement of health technology assessment (HTA) models to focus on one disease. Using Cardiff, a model previously built for type 2 diabetes (T2D), we illustrate the changes needed to capture contemporary, holistic, patient-centered decision-making, and argue that HTA bodies should revise their approach.
Methods: The upgraded model enables therapy selection and escalation determined by HbA1c thresholds, cardiovascular risk (QRISK3), comorbidities (established cardiovascular or chronic kidney disease), and weight (body mass index ≥35 kg/m^2). Risk factor trajectories were updated by incorporating UKPDS-90 equations and other relevant data sources. Clinical outcomes were predicted using new risk equations incorporating cardiovascular outcomes trial data whenever possible. The updated model was applied to assess quality-adjusted life years (QALYs) and lifetime costs in newly diagnosed T2D patients in the UK, modeled via a conventional glycemic-centric approach versus a multifactorial treatment algorithm. Extrapolation to the national level utilized estimates of annual incidence.

https://doi.org/10.1017/S0266462324000667