Living health technology assessments: how close to living reality?

Grammati Sarri 1, Anna Forsythe, 2 Jamie Elvidge, 3 Dalia Dawoud 3

Introduction

Healthcare decision-makers are exploring more responsive, innovative processes in the wake of the COVID-19 pandemic, including a ‘living’ approach to health technology assessment (HTA).  Even before the pandemic, the use of real-world data (RWD) and the advent of mobile and digital health technologies were transforming HTA decision making.  These developments coincided with a broad recognition that keeping pace with rapid publication of new evidence and variation/inefficiencies in review can lead to HTA decisions based on out-of-date evidence.  These challenges hinder timely patient access to promising, innovative health technologies when decision-makers are asked to accept higher uncertainty in the evidence base, especially in populations with substantial unmet need.  More reactive and flexible ‘living’ approaches to HTA should be explored.

This commentary outlines challenges of current, ‘static’ HTA approaches, offers solutions provided by a ‘living’ HTA approach, and considers implementation of this method.

Are current HTA approaches optimal to address ongoing evidence generation?

HTA processes have remained largely unchanged, but innovative study designs (eg, pragmatic and adaptive clinical trials, single-arm trials) are transforming evidence generation, while regulatory decisions are increasingly being based on surrogate endpoints rather than primary outcomes.  This introduces uncertainties which require supplemental data to validate additional assumptions in the analyses.  Recently, managed entry arrangements were developed to regulate reimbursement of new technologies with promising but uncertain benefits.  For example, coverage with evidence development (CED) schemas grant patients temporary access to novel treatments while additional evidence is systematically collected.  Decision making based on CED, however, largely ignores a technology’s ‘lifecycle’ in the context of evolving evidence.  A 2019 review of CED decisions in Netherlands highlighted how systematically identifying uncertainty can guide the feasibility of follow-up evidence generation.  A 2022 review of economic models from National Institute for Health and Care Excellence (NICE) technology appraisals in England showed how uncertainty caused by unsupported predictors, use of surrogate outcomes and lack of a model’s transparency can be overcome by regular technology reassessment.  In Sweden, evidence generated from the CED for a novel treatment for advanced Parkinson’s disease was unconvincing during HTA reassessment, causing it to be withdrawn from clinical practice; reimbursement was reinstated after a re-evaluation of follow-up data.

The lack of periodic re-evaluation of technologies unnecessarily strains overburdened healthcare systems which miss opportunities to disinvest in technologies that do not maintain their value or reconsider the value of approved technologies in light of new evidence.

Is ‘living’ HTA a viable solution to accommodate continual evidence generation, assessment and decision making?

Living HTA is a real-time, dynamic approach that uses explicit methods to determine the value of a health technology at different points in its lifecycle from the point of use (market access) through continued evidence generation.  This can resolve some common uncertainties and evidence gaps seen in initial HTA submissions (especially close to product launch) related to the target population, disease, costs or the new therapy (eg, adding subgroups, real-world effectiveness and safety, validating surrogate outcomes, survival extrapolations and economic data).  New safety evidence can also inform updated decision making.  Living systematic reviews (LSR) which combine contemporaneity and rigour to enhance the data accuracy and utility for decision making are now widely accepted as an alternative to traditional single, static reviews and living, adaptable whole-disease pathway economic models can inform pricing renegotiations.

There has been an explosion of technological applications in evidence synthesis, data analysis and economic modelling, and the integration of automation is central to this living process.  Methods to trigger systematic review updates have been proposed and artificial intelligence tools (eg, machine learning algorithms) were tested in specific applications in comparative effectiveness research.  These tasks are resource intensive but can be significantly streamlined through automation.  Similar developments have been proposed in economic modelling.

Correspondence to: Dr Grammati Sarri, Cytel Inc, Waltham, London, UK; grammati.sarri@cytel.com

© Author(s) (or their employer(s)) 2023. Re-use permitted under CC BY-NC. No commercial re-use. See rights and permissions. Published by BMJ.

To cite: Sarri G, Forsythe A, Elvidge J, et al. BMJ Evidence-Based Medicine Epub ahead of print: [please include Day Month Year]. doi:10.1136/bmjebm-2022-112152
How can ‘living’ HTA be implemented?

Pre-existing conceptual frameworks for lifecycle HTA processes can provide the basis for living HTA implementation by incorporating: (1) transparent processes on safe integration of digital tools (eg, frequency of and trigger for review); (2) presentation of updated LSR and modelling results; and (3) updated reporting guidance. Fundamentals for automation, continuous improvement and maintaining high-quality standards have been covered in the emerging literature on this topic. One study determined LSRs were a suitable approach during a pandemic, and outlined methodological challenges that may inform future research. Other structured frameworks for integrating RWD in evidence synthesis can help implementation of a living HTA approach. Technological aversion—a long-standing obstacle to innovations in healthcare decision making—can be overthrown by open dialogue, collaboration and standardisation of processes, as well as targeted training by HTA agencies to upskill their staff. Issues around data privacy, transparency, access and validation of operational procedures are key to support development of living HTA frameworks.

A living HTA process may standardise search strategies, data extraction templates, review methodologies and modelling approaches, and will help eliminate duplication of efforts across HTA agencies. An online platform (MAGICapp) used during the recent pandemic for uploading living guidelines and encouraging continuous improvement and maintaining high-quality standards have been covered in the emerging literature on this topic. One study determined LSRs were a suitable approach during a pandemic, and outlined methodological challenges that may inform future research. Other structured frameworks for integrating RWD in evidence synthesis can help implementation of a living HTA approach. Technological aversion—a long-standing obstacle to innovations in healthcare decision making—can be overthrown by open dialogue, collaboration and standardisation of processes, as well as targeted training by HTA agencies to upskill their staff. Issues around data privacy, transparency, access and validation of operational procedures are key to support development of living HTA frameworks.

A living HTA process may standardise search strategies, data extraction templates, review methodologies and modelling approaches, and will help eliminate duplication of efforts across HTA agencies.

Cost implications of HTA must be proportionate to the expected societal benefit including the cost of potentially inappropriate commissioning decisions. Automation of evidence retrieval, screening, data collection and analyses can minimise any economic burden of a living HTA approach. The Australian Living Stroke Clinical Guidelines found a 99% reduction in time from research to point of care with significant savings to multiple stakeholders. However, the living HTA approach should be piloted to assess its pros and cons. Health system resources and country-specific priorities will determine if a living appraisal framework is more appropriate in certain cases, such as high-cost or high-impact technologies, innovations that are CED candidates or in diseases with rapidly evolving treatment landscapes.

Next steps?

HTA bodies can embrace a living approach by enhancing their technological capabilities and framework structures. Recent efforts to harmonise and streamline HTA processes at a regional and cross-border level (eg, the proportionate approach to HTA in the UK by NICE, the European Union (EU) Joint Clinical Assessments, the Access Consortium, The Innovative Licensing and Access Pathway and Project Orbis) will help HTA bodies to manage workloads and reduce duplicative efforts, allowing living HTA to become a living reality.

Acknowledgements The authors would wish to thank Colleen Dumont for providing editorial support for this manuscript.

Contributors GS developed the scope for this manuscript. All coauthors contributed to the scope of the manuscript and provided detailed comments on prepared versions. The final version of this article was approved by all authors.

Funding The authors have not declared a specific grant for this research from any funding agency in the public, commercial or not-for-profit sectors.

Disclaimer The views expressed are those of the authors and not those of their respective organisations.

Competing interests GS and AF are employed by Cytel Inc. JE and DD are employed by NICE.

Patient consent for publication Not applicable.

Provenance and peer review Not commissioned; externally peer reviewed.

Open access This is an open access article distributed in accordance with the Creative Commons Attribution Non Commercial (CC BY-NC 4.0) license, which permits others to distribute, remix, adapt, build upon this work non-commercially, and license their derivative works on different terms, provided the original work is properly cited, appropriate credit is given, any changes made indicated, and the use is non-commercial. See: http://creativecommons.org/licenses/by-nc/4.0/.

ORCID iD Grammati Sarri http://orcid.org/0000-0001-5536-8038

References


16 Smith RA, Schneider PP, Mohammed W. Living health economic evaluation with R [Internet]. *Wellcome Open Res* 2022;7:194. 10.12688/wellcomemar.17931.2 Available: https://wellcomeopenresearch.org/articles/7-194


