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VALUE & OUTCOMES SPOTLIGHT

A magazine for the global HEOR community.

UNLOCKING AFFORDABILITY & ACCESS WITH HEOR

ISPOR
Improving healthcare decisions

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MARCH/APRIL 2025
VOL. 11, NO. 2

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A magazine for the global HEOR community.

The mission of *Value & Outcomes Spotlight* is to foster dialogue within the global health economics and outcomes research (HEOR) community by reviewing the impact of HEOR methodologies on health policy and healthcare delivery to ultimately improve decision making for health globally.

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Global Health Equity: Strategies for Improving Affordability and Access

In the ever-evolving landscape of global healthcare, affordability and access to essential treatment and care remain pivotal themes. The disparity in healthcare access between developed nations and low- and middle-income countries (LMICs) underscores the urgent need for innovative pricing strategies, tiered pricing, donations, robust healthcare infrastructure, capacity building, and partnerships with local governments and nonprofits to ensure equitable health outcomes for all.

In developed countries, pharmaceutical companies often employ tiered pricing strategies to enhance medication affordability for underinsured or low-income populations. This approach not only improves health outcomes but also fosters a more inclusive healthcare system. By adjusting prices based on the economic status of different population segments, these companies can make life-saving medications accessible to a broader audience, thereby reducing healthcare disparities within these nations.

Conversely, in LMICs, the economic constraints are more pronounced, necessitating even more significant interventions to ensure medication affordability. Special pricing strategies in these regions often include substantial price reductions, donations, or the provision of generic alternatives. These measures are crucial in bridging the healthcare gap and promoting global health equity. By making essential medications affordable and accessible, pharmaceutical companies and healthcare organizations can significantly improve the quality of life for vulnerable populations in these countries.

> By evaluating the economic, clinical, and humanistic outcomes of healthcare practices, HEOR provides robust evidence of a treatment's value beyond its efficacy.

Efforts to provide medications to patients in LMICs are accelerating health equity through various initiatives. Tiered pricing, donations, and partnerships with local governments and nonprofits play a vital role in making essential medications more affordable and accessible. These measures address significant healthcare disparities by ensuring that even the most vulnerable populations receive necessary treatments. Additionally, programs focused on strengthening healthcare infrastructure and increasing local capacity for drug manufacturing further support sustainable access. As a result, these efforts are closing the health equity gap, leading to improved health outcomes and a higher quality of life in LMICs.

Different healthcare systems address the challenge of "rationing" in various ways to manage limited resources effectively. In publicly funded systems, such as the United Kingdom's National Health Service, rationing is often achieved through cost-effectiveness evaluations by bodies like the National Institute for Health and Care Excellence. These evaluations prioritize treatments that offer the most benefit relative to cost, ensuring that resources are allocated efficiently. In contrast, private healthcare systems, like those in the United States, may ration through insurance coverage decisions, where insurers determine which treatments are covered based on policy terms and cost considerations. Additionally, some countries employ waiting lists to manage demand for services, ensuring that urgent cases receive priority. These approaches aim to balance resource constraints with equitable patient care.

Demographic changes, particularly an aging population, will significantly impact the prevalence of chronic health problems such as obesity, diabetes, and rare diseases. As the elderly population grows, the demand for healthcare services will increase, straining

existing healthcare systems. This will likely lead to higher healthcare costs, impacting affordability. Additionally, increased demand may lead to longer wait times and potential shortages of healthcare providers, affecting access to care. To address these challenges, healthcare systems must innovate, potentially through telemedicine, improved chronic disease management, and policies that ensure equitable access and affordability for all, especially vulnerable aging populations.

> Healthcare accessibility and affordability are critical for effective health systems, yet barriers persist globally.

Health economics and outcomes research (HEOR) plays a pivotal role in supporting the value story of medical treatments and interventions. By evaluating the economic, clinical, and humanistic outcomes of healthcare practices, HEOR provides robust evidence of a treatment's value beyond its efficacy. This research helps demonstrate cost-effectiveness, improve patient quality of life, and inform healthcare decision making. By quantifying benefits such as reduced hospitalizations, improved patient adherence, and long-term health gains, HEOR strengthens the case for adopting new therapies. Ultimately, HEOR helps stakeholders, including payers, providers, and policy makers, make informed decisions that enhance patient care and optimize resource allocation.

Healthcare accessibility and affordability are critical for effective health systems, yet barriers persist globally. High-income countries strive to balance comprehensive care and cost-effectiveness, while LMICs face significant challenges in accessing essential drugs and adequate reimbursement mechanisms. International collaboration, ethical responsibility, and innovative strategies are essential to address these disparities and promote global health equity. By leveraging HEOR and other initiatives, we can enhance access to healthcare, improve quality of life, and ensure a healthier future for everyone.

As always, I welcome input from our readers. Please feel free to email me at zeba.m.khan@hotmail.com.

Zeba M. Khan, RPh, PhD
Editor-in-Chief,
Value & Outcomes Spotlight

FROM THE CEO

Enhancing the Accessibility and Affordability of Healthcare

Rob Abbott, CEO & Executive Director, ISPOR

Anyone who cares about healthcare—and who looks objectively at the current state of healthcare systems around the world—knows that accessibility and affordability are huge barriers that create pervasive health inequities. Globally, nearly 2 billion people have no access to essential medicines—and these challenges of accessibility and affordability aren't limited to low- and middle-income countries (LMICs). In the United States, many Medicare beneficiaries don't even fill their prescription or skip taking it because of the expense. The same is true in Canada. Access to affordable, high-quality medicines is crucial to reducing unnecessary pain and suffering, shortening the duration of illness, and addressing the financial burden of care worldwide. All of these elements are embodied in ISPOR's vision of a world where healthcare is accessible, effective, efficient, and affordable for all.

> Access to affordable, high-quality medicines is crucial to reducing unnecessary pain and suffering, shortening the duration of illness, and addressing the financial burden of care worldwide.

I'm therefore proud that this issue of *Value & Outcomes Spotlight* focuses squarely on the twin issues of accessibility and affordability. Too often, we become excited over the arrival of a new treatment (gene therapies come to mind) without considering the cost, or we assume that conditions of patient access in other parts of the world mirror those in our home country. As the articles in this issue make clear, accessible and affordable care is a common challenge around the world.

This challenge is most acutely felt in LMICs, resulting in profound health inequities. Far too many people in LMICs don't have access to essential healthcare services, and this lack of access is exacerbated by social determinants of health like poverty, entrenched discrimination, gender inequality, and climate change, to cite some of the more prominent examples. Further, the rise of noncommunicable diseases such as cancer and heart disease, coupled with the ongoing burden of infectious diseases, is stressing already fragile health systems to the breaking point. The World Health Organization estimates that more than three-quarters of global deaths due to noncommunicable diseases occur in LMICs. The lack of access to medicines and diagnostics only compounds the problem and creates an unacceptable social opportunity cost. I have a chart in my office showing life expectancy at birth for every country on Earth. The contrast between high-income and low-income countries is stark. At the extreme (Monaco versus Afghanistan), the gap can be as much as 30 years.

The news is not all bad; several pharmaceutical companies have made genuine efforts to prioritize LMICs within their business models, but the pace of progress is slow. The 2024 Access to Medicine Index, which ranks 20 of the world's largest pharmaceutical companies based on their efforts to improve access to essential medicines in LMICs, showed that current efforts are falling short. In particular, momentum in licensing activity has stalled, with only 2 new nonexclusive voluntary licensing agreements identified in the 2024 Index, compared with 6 in 2022. More broadly, there is a gap in clinical trial activity in LMICs. Roughly 40% of clinical trials take place in the 113 LMICs covered by the Index analysis, despite being home to 80% of the global population. The fate of healthcare in LMICs is not a problem to be solved exclusively by pharmaceutical companies, but it is a future to be created through multistakeholder collaboration. ISPOR stands ready to build on our previous efforts in LMICs, leveraging our network of chapters across more than 100 countries, to assist in this critical work.

Closer to home, the Inflation Reduction Act of 2022 included several provisions to make prescription drugs more affordable and accessible for Medicare beneficiaries, most notably putting a $2000 yearly cap on beneficiaries' out-of-pocket costs for Part D prescription drugs. A key feature of the law, of course, is the ability of the US government to negotiate drug prices for Medicare. The first 10 price-protected drugs—including the blood thinner apixaban (Eliquis) and the diabetes medicine sitagliptin (Januvia)—take effect in 2026. While it is not yet clear how the new administration in Washington, DC will approach future negotiations, one can only hope that more drugs will be added to this list each year. Still, with more than 20,000 approved drugs on the market, other steps are needed to improve accessibility and affordability.

> I have a chart in my office showing life expectancy at birth for every country on Earth. The contrast between high-income and low-income countries is stark. At the extreme (Monaco versus Afghanistan), the gap can be as much as 30 years.

With the above in mind, our field of health economics and outcomes research (HEOR) has much to offer in the search for solutions that enhance accessibility and affordability. It's worth remembering that HEOR emerged as a combination of 2

separate fields. Health economics focuses on determining the value of medical interventions while also considering broader issues such as the overall healthcare market and available healthcare funding. Outcomes research focuses on the results of medical interventions *as observed by actual patients*. This latter point is crucial; the evidence that HEOR compiles is different from clinical trial data because it is based on real-world clinical, health, and economic issues encountered by patients and healthcare providers. It is here that we have an opportunity to engage first-hand with patients, especially in LMICs, to grow our collective understanding of the challenges associated with accessibility and affordability. I should add that ISPOR's Global Access to Medical Innovation special interest group has a twofold purpose: (1) to educate and inform ISPOR members and stakeholders on the basics of global access, especially as it relates to the role of HEOR across health systems, as well as new methodological and policy developments; and (2) to leverage the high motivation and skills of ISPOR's global community of researchers, patient representatives, and other life science professionals to improve global access to medical innovation while stimulating its production.

There is much to be done to make progress toward ISPOR's vision, and to address some of the challenges highlighted in the articles in this issue of *Value & Outcomes Spotlight*. Our new 2030 strategy provides a roadmap for how we can make progress over the next 5 years. As your CEO, I pledge to work with my staff, my Board, ISPOR's incredible membership, and our collaborating partners around the world to make it happen.

ISPOR NEWS

ISPOR Embraces Open Access

Earlier this year, ISPOR made the decision to transition *Value in Health Regional Issues* to an open access journal. This change marks a pivotal moment in the dissemination of health economics and outcomes research (HEOR), making it freely available to readers worldwide.

The shift to open access represents more than just a change in publication policy—it's a fundamental reimagining of how scientific knowledge is shared and used. With the transition to open access, ISPOR has removed the paywall that previously restricted access to valuable research, effectively providing universal access to everyone in the world.

Why is this important?
The move to open access is part of a broader trend in academic publishing. This shift reflects a growing recognition of the importance of open science and the need for equitable access to research findings, especially in fields with significant public health and public policy implications.

ISPOR's decision to transition *Value in Health Regional Issues* to an open access journal represents a significant step forward in the dissemination of HEOR in emerging markets. The shift aligns with ISPOR's mission to promote HEOR excellence to improve decision making for health globally and strengthens the journal's ability to inform decisions, shape policies, and inspire new research directions across regional geographies.

Key benefits of the transition

1. Initial Fee Waiver: To ease the transition, all article-processing charges will be waived for papers submitted through December 31, 2025.
2. Increased Visibility: Open access significantly expands the discoverability and potential readership of published studies, driving citations and overall impact for authors.
3. Equitable Access: This model promotes fairness by allowing researchers, students, and policy makers from all economic backgrounds to access vital health economic research.

The broader impact
The impact of this change extends beyond academia, promising to inform public discourse and provide global health insights. Expected outcomes include enhanced public understanding of HEOR, innovation in healthcare delivery and policy, and faster research advancements.

By removing barriers to access, ISPOR hopes to empower researchers, regulators, policy makers, and healthcare professionals worldwide to make more informed decisions based on the latest regional insights and analyses. As *Value in Health Regional Issues* enters this new era, it stands poised to make an even greater impact on the understanding and application of HEOR around the world.

Disclosure: *This content was created with assistance from artificial intelligence (AI). The content has been reviewed and edited by ISPOR staff. For more information on ISPOR's AI policy, click here.*

ISPOR Conferences and Events

ISPOR 2025 | Tuesday, May 13 - Friday, May 16

Montréal Convention Centre | Montreal, QC, Canada

ISPOR 2025, is just weeks away—don't miss out! This year's International Conference is fast approaching! With the theme "Collaborating to Improve Healthcare Decision Making for All: Expanding HEOR Horizons," ISPOR 2025 will feature insightful sessions covering healthcare policy, health technology assessment, real-world evidence, patient-centered research, digital health, health equity, and value assessment.

Join leading experts as they explore the evolving role of health economics and outcomes research (HEOR) in shaping innovation, value, and global healthcare decision-making—both today and in the future.

Don't wait—explore the program, book your hotel , and secure your spot today!

Have you viewed our recently announced plenary sessions?

May 14 – Plenary 1: **Drug Price Controls—What Are the Unintended Consequences to Innovation?**

May 15 – Plenary 2: **Evolution of Evidence—Innovating for the Future of HTA**

May 16 – Plenary 3: **Balancing Speed and Scientific Rigor—Patient-Centered Methodologies for Surrogate Endpoints in Accelerated Access**

The following is a sampling of conference sessions that align with the top 3 ranked hot topics in the ISPOR 2024-2025 Top 10 HEOR Trends Report:

#1 Real-World Evidence

Real-World Life-Cycle Evaluation for Precision Medicine: From Conceptualization to Successful Implementation | *Breakout Session* | *May 14*

#2 Drug Pricing

Medicare Price Negotiation of Part B Drugs: Implications for Provider Reimbursement and Commercial Spillover | *Spotlight* | *May 14*

#3 Artificial Intelligence

Prompt Engineering: Harnessing Generative Artificial Intelligence for HEOR | *Spotlight* | *May 15*

Prepare for the deeper-dive conference sessions with pre-conference short courses

A full day of short courses will be held on May 13. The ISPOR Short Course Program is designed to enhance knowledge and techniques in core HEOR topics as well as emerging trends in the field. Taught by expert faculty, courses span across 7 topical tracks and range in skill level from introductory to experienced.

More at www.ispor.org/ISPOR2025

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ISPOR Conferences and Events

ISPOR Real-World Evidence Summit 2025:
Through the Lens of Asia Pacific | 28-30 September

Tokyo Prince Hotel | Tokyo, Japan

Save the date for the **ISPOR Real-World Evidence Summit 2025: Through the Lens of Asia Pacific**. Join us in Tokyo for exclusive access to the latest advancements in real-world evidence methodologies, data analysis, and applications designed to solve the region's most pressing healthcare challenges, shared by top experts, decision makers, and industry leaders. The 3-day summit will present learning opportunities that range from plenary sessions to short courses, breakout sessions to educational symposia, posters, and more! View the preliminary program here.

More at www.ispor.org/Summit2025-RWE

Connect with colleagues across the region using #ISPORSummit

ISPOR Europe 2025 | 9-12 November
Scottish Event Campus | Glasgow, Scotland, UK

Share your expertise at ISPOR Europe 2025. Abstract submissions are now open!

ISPOR Europe 2025 is calling for abstract submissions for issue panels, workshops, breakout sessions, and HEOR impact cases! This is your chance to present your research to a global audience and connect with fellow HEOR professionals in the vibrant city of Glasgow.

As a must-attend event, ISPOR Europe 2025 brings together researchers, academicians, regulators, payers, policymakers, life sciences professionals, healthcare providers, and patient engagement organizations—all dedicated to advancing healthcare decision making.

Research abstract submissions open 17 April—don't miss this opportunity! Get the details and submit your abstract.

More at www.ispor.org/Europe2025

Join the conversation on social media using #ISPOREurope

Learn more about sponsorship opportunities for the ISPOR Real-World Evidence Summit 2025 and ISPOR Europe 2025. For inquiries reach out to sales@ispor.org.

ISPOR Education

ISPOR Short Courses

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April 23-24 | Virtual

Introduction to Machine Learning Methods

This intermediate-level course will offer hands-on experience with machine learning for healthcare data analysis.

June 11-12 | Virtual

Introduction to Health Technology Assessment

This introductory-level course will lay the foundation of health technology assessment to benefit your informed decision making.

Explore in-person short courses at ISPOR 2025 here, and browse the full list of upcoming short courses at www.ispor.org/shortcourses.

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- 25% discount for members on open access publishing fees for *Value in Health*
- Access to member groups (eg, special interest groups, task force review groups, chapters)
- Networking through online member communities and volunteer opportunities
- Discounts on conference, Summit, short course, and ISPOR Education Center course registrations

Go to **ISPOR.org** today to join or renew your membership.

ISPOR Education

ISPOR Webinars

April 23

Causal AI in HEOR: Beyond Predictions to Actionable Insights

Presented by the ISPOR Student Network Education Committee, this webinar focuses on the fundamentals and transformative role of causal artificial intelligence as it relates to HEOR.

June 10

ADHD, GLP-1s, & HIV: Real-World Trends in Prescribing and Adherence

Hear key insights from 4 new studies to be presented at ISPOR 2025, demonstrating how data can quickly be uncovered in real-world practice patterns for high-profile therapies.

Sponsored by Corporate Partner, Truveta.

June 12

ISPOR HEOR Competencies: Boosting Career and Networks

Presented by ISPOR Global Groups, this webinar will define the list of competencies necessary for professionals to excel in the field of HEOR.

June 17

Why Elicit Utility Weights for Cost Effectiveness Analysis Using DCEs?

Gain an introduction to the methods and applications of discrete choice experiments for eliciting utilities for cost-effectiveness analyses in this webinar. Presented by the ISPOR Patient-Centered Special Interest Group.

June 18

Negative Control Outcomes in Observational Studies of Effectiveness

This webinar will focus on the use of negative control outcomes as a mechanism to evaluate confounding before embarking on a comparative analysis. Presented by the ISPOR Statistical Methods in HEOR Special Interest Group.

View all upcoming and on-demand webinars at www.ispor.org/webinars

ISPOR Education

ISPOR Education Center

Immerse yourself in learning with the ISPOR Education Center, where courses range from 1-4 hours and are available at a time that works for you. Complete the course to earn an ISPOR Education Badge and an ISPOR Certificate of Completion.

The following are the newest courses in the catalog:

Introduction to Implementation Science

Develop plans for conducting, executing, and evaluating an implementation science study. Utilizing key learned principles, you will design your own implementation science study.

Environmental Sustainability in Health Technology Assessment

Integrating sustainability into health technology assessment is critical as healthcare decision making evolves to address environmental challenges. This course introduces the principles of sustainable healthcare and methods for assessing environmental impacts of health technologies.

View the growing catalog of courses at www.ispor.org/EducationCenter

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Catch up or revisit recorded educational content from past ISPOR conferences and summits in the HEOR Learning Lab™. Content is focused on the most topical themes impacting the field, including real-world evidence, patient-centered research, digital health, artificial intelligence and machine learning, health technology assessment, economic methods, healthcare financing, access and policy, learning healthcare systems, and much more.

The following are examples of popular sessions available for viewing:

Getting Real About Real-World Data From Electronic Health Records

Explore the factors that impact RWE generated from electronic health record (EHR) data, and how challenges can be overcome to improve the uses and reliability of EHR data for research and regulatory purposes.

AI Enabling Whole Health: Opportunities and Challenges for HEOR and HTA

Gain insight into the future of AI and its impact on whole health with this plenary session from ISPOR 2024.

Advanced Methods for Matching-Adjusted Indirect Comparison

Review the main advantages and limitations of matching-adjusted indirect comparisons and get updated on newly proposed methods.

View more sessions at www.ispor.org/LearningLabWelcome

FROM THE REGIONS

The Importance of Global Collaboration and Knowledge Exchange: A Summary Report of the Global ISPOR HTA Roundtable

Kelly Lenahan, MPH, ISPOR, Lawrenceville, NJ, USA; **Jessica Daw, PharmD, MBA**, Pharmacy, Sentara Health Plans, Virginia Beach, VA, USA; **Mitch Higashi, PhD**, ISPOR, Lawrenceville, NJ, USA

Health technology assessment (HTA) bodies and health insurance organizations/payers globally are increasingly being asked to assess innovative health technologies as new drug approvals are on the rise.[1,2] The rapid development and introduction of new treatments, particularly advanced therapy medicinal products (ATMPs), gene therapies, precision medicines, and other advanced therapies, present significant challenges for these HTA bodies. These challenges include limited evidence at the time of approval, uncertainty in long-term outcomes, high costs, affordability concerns, equity in access to treatments, and sustainability of healthcare systems. The unique characteristics of advanced therapies, such as their high upfront costs and potential long-term benefits, make assessing their value and affordability particularly difficult, with an additional burden for low- and middle-income countries (LMICs). These countries often face greater difficulty in financing and accessing these high-cost technologies, raising important questions about global equity and healthcare system sustainability.

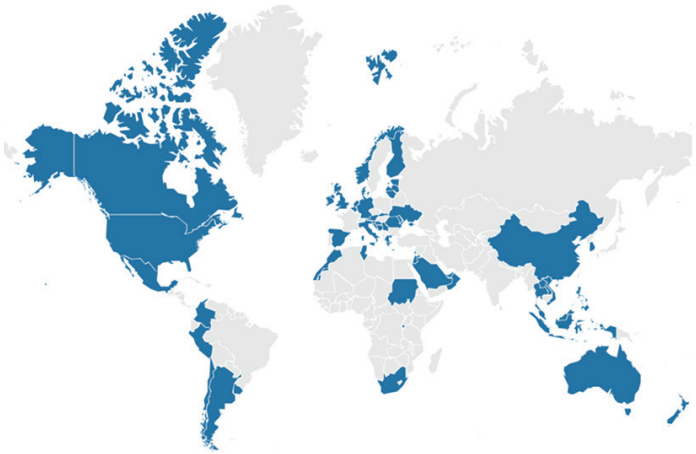
> To improve methodology and information sharing between HTA bodies, ISPOR has been holding HTA Roundtables in 5 major regions of the world annually since 2007.

HTA is a multidisciplinary process that uses systematic and explicit methods to evaluate the properties and effects of a health technology.[3] A health technology is an intervention developed to prevent, diagnose, or treat medical conditions; promote health; provide rehabilitation; or organize healthcare delivery. The intervention can be a test, device, medicine, vaccine, procedure, program, or system.[4] To improve methodology and information sharing between HTA bodies, ISPOR has been holding HTA Roundtables in 5 major regions of the world annually since 2007 (Asia Pacific, Europe, Latin America and the Caribbean, Middle East and Africa, and North America). These meetings are comprised of HTA bodies, payers and health insurance organizations, other governmental decision makers such as Ministries of Health or the World Health Organization, and academics if no HTA body exists in a country/jurisdiction. However, these meetings have always taken place regionally and not on a global scale. Throughout the years of holding HTA Roundtables, ISPOR continued to hear the need for cross-regional discussion and information sharing. Therefore, for the first time in the 17-year history of the ISPOR HTA Roundtables, ISPOR held the Global HTA Roundtable on October 23, 2024, to bring together the 5 regions to share information and learn from one another.

One hundred participants from 53 countries/jurisdictions globally attended the virtual meeting (**Figure**). A pre-event survey found 3 major aspects of innovative health technologies that participants wanted to discuss: (1) ATMPs/gene therapies/precision medicine, (2) digital health, and (3) high-cost technologies. ATMPs and gene therapies represent a paradigm shift in treatment approaches, often offering potential cures for previously untreatable conditions. These therapies also come with significant challenges in terms of evidence generation, pricing, and healthcare system readiness. High-cost drugs for rare diseases pose unique challenges due to small patient populations and limited evidence, while those for prevalent diseases raise concerns about budget impact and system sustainability. Digital health technologies and artificial intelligence present novel challenges in terms of evidence standards, privacy concerns, and integration into existing healthcare systems. These topics highlight the need for HTA methodologies to evolve to assess these diverse and complex innovations adequately.

An overview of the current situation regarding the assessment of these technologies in each region was presented; then participants were divided into 6 breakout groups to discuss the successes and opportunities for each of the 3 major topics listed above. An online survey tool was used to collect responses from attendees for each breakout group. Items identified during the breakouts were then voted on by the entire group. The results of the discussion are below.

Figure. Geographic representation of attendees of ISPOR's HTA Roundtables.



ATMPs and Gene Therapies

ATMPs, including gene therapies, represent cutting-edge treatments with the potential to provide curative solutions for previously untreatable conditions. However, these therapies often come with extremely high costs, sometimes reaching millions of US dollars per patient, which places a significant strain on healthcare systems. This cost challenge is exacerbated by the limited available evidence at the time of approval, as well as the small patient populations for many rare diseases. The complexity and high cost of these treatments make it difficult for HTA bodies to assess their value and affordability, particularly in countries with limited resources.

> There is a strong call for better collaboration between HTA bodies and payers to ensure price transparency and for the development of global frameworks tailored to assessing ATMPs.

A key concern raised during the roundtable discussions was the significant global disparities in financing and access to ATMPs. For instance, in the United States and Belgium, innovative financing mechanisms, such as value-based contracting and companion diagnostics, have been implemented, but other countries, like Morocco, finance gene therapies on a case-by-case basis. This disparity in access highlights the need for innovative financing models and greater international collaboration to address global inequities in healthcare access.

To reduce costs, European hospitals have expressed interest in developing on-site production for chimeric antigen receptor T-cell therapies (CAR-Ts). On-site production would reduce treatment delays, lower costs, and allow for more personalized development of the technology; however, many regulatory challenges face this manufacturing change due to the complex nature of CAR-Ts. Additionally, joint price negotiations, as seen in the BeNeLuxA initiative, offer a strategy for smaller countries to increase bargaining power and potentially secure more favorable pricing terms. In the United States, value-based contracting for gene therapies is gaining traction, with contracts often extending for several years to ensure the effectiveness of the treatment.

Another challenge facing ATMPs/gene therapies is the limited number of specialized treatment facilities. This is particularly true for newer therapies like those for sickle cell disease. The complex nature of these treatments often requires highly specialized centers with specific expertise and equipment. For example, in the United States, CAR-T cell therapies are typically only available at select cancer centers. This limitation can create significant geographic and logistical barriers for patients who have to travel many hours to receive treatment, leading to potential health disparities. Access issues also impact utilization rates, with some therapies seeing lower uptake than initially anticipated. This situation highlights the need for infrastructure development and healthcare workforce training to expand access to these innovative treatments.

Overall, the Global HTA Roundtable came to consensus that the global HTA community is succeeding by relying on evidence when making reimbursement and coverage decisions and funding through outcomes-based contracts/managed entry agreements. However, there is a strong call for better collaboration between HTA bodies and payers to ensure price transparency and for the development of global frameworks tailored to assessing ATMPs.

Digital Health Technologies and Artificial Intelligence

Digital health technologies, including artificial intelligence (AI)-enabled systems, present unique challenges for HTA. The rapid pace of technological development and the diverse range of digital interventions make it difficult to create standardized assessment frameworks. Moreover, the dynamic nature of AI, which can learn and evolve over time, adds complexity to its evaluation. Some HTA bodies have developed frameworks to assess these technologies, but they must continually adapt to keep pace with innovation.

Table. References shared at the Global HTA Roundtable related to the assessment of digital health technologies and artificial intelligence.

Country/Region	Organization	Title	Reference
United States	Institute for Clinical and Effectiveness Review (ICER) and Peterson Health Technology Institute (PHTI)	Framework for Digital Health Technologies	5
Spain	Agència de Qualitat i Avaluació Sanitàries de Catalunya (AQuAS)	Health Technology Assessment Framework: Adaptation for Digital Health Technology Assessment	6
Europe	European Digital Health Technology Assessment Project (EDiHTA Project)	European Digital Health Technology Assessment Framework	7
United Kingdom	National Institute for Health and Care Excellence (NICE)	Evidence standards framework (ESF) for digital health technologies	8
United Kingdom	National Institute for Health and Care Excellence (NICE)	Use of AI in evidence generation: NICE position statement	9
Asia Pacific and Europe	The Asia Pacific Medical Technology Association (APACMed)	The Comparison Analysis of HTA Guideline for Digital Health Technologies (DHTs) in Korea, United Kingdom, France, and Germany	10
Canada	Canada's Drug Agency (CDA-AMC)	RapidAI for Stroke Detection: Main Report	11

AI presents particular challenges related to transparency, the need for specialized expertise, and the integration of AI algorithms into healthcare systems. Many countries are cautiously optimistic about the potential of AI to improve HTA processes, particularly by automating routine tasks and enhancing efficiency. However, there are concerns about the risk of AI exacerbating equity issues between high-income countries (HICs) and low- and middle-income countries (LMICs), as limited data from the latter can lead to biases in AI systems. Furthermore, there is a need to ensure the quality of the data used by AI systems, as inaccurate or incomplete data could undermine the effectiveness of these technologies.

Overall, the Global HTA Roundtable agreed that the HTA community is being successful by being cautious, but open minded, about the use of AI in HTA. The biggest opportunity for improving how the HTA community assesses digital health and AI is the need for standardization of guidelines and methods relating to these types of technologies, including more information sharing of best practices and current guidelines.

High-Cost Technologies

The assessment of high-cost technologies, particularly those used for rare diseases, is another area of significant concern. Innovative payment models, such as pay-for-performance and outcomes-based contracts, have emerged as potential solutions to manage the costs of these treatments. However, these models face significant challenges in terms of implementation, including difficulties in determining when to withdraw treatments that do not meet performance criteria and the need for transparent communication among stakeholders.

In Europe, there has been a shift toward assessing the real clinical benefit of technologies rather than just their cost. This approach aims to prioritize technologies that provide significant patient benefits, particularly in cases where the technology is highly innovative. Canada's "Drugs for Rare Diseases Strategy" is another example of a proactive approach to managing high-cost treatments, with a financial commitment to subsidizing rare disease drugs and collecting real-world evidence to assess their long-term effectiveness.

> The HTA community is being successful by being cautious, but open minded, about the use of AI in HTA.

Ukraine has successfully implemented Managed Entry Agreements (MEAs) for high-cost drugs. In 2022, 9 medicines went through financial-based agreements, resulting in over $10 million in savings for the healthcare system. These savings have, in turn, enabled access to new therapies that might otherwise have been unaffordable.[12] Other countries in central and eastern Europe, such as Moldova, also plan to implement MEAs in the next year.

Argentina's prioritization system for high-cost drugs, which considers factors such as equity and alignment with public health priorities, is another example of how countries are addressing these challenges. Argentina's system gives higher priority to technologies that have already been evaluated by agencies in other countries, such as NICE in the United Kingdom or Canadian agencies. This approach allows Argentina to leverage international expertise and streamline its own assessment process, which is particularly valuable given the country's resource constraints.

> There is a strong need for information sharing across countries and regions to learn what others are doing and how to share best practices.

Overall, the Global HTA Roundtable came to consensus that the HTA community is succeeding by using price negotiations, managed entry agreements, and using cost-effectiveness thresholds. There is a strong need for information sharing across countries and regions to learn what others are doing and how to share best practices.

Conclusion

The Global HTA Roundtable highlighted the need for greater international collaboration and information sharing to address the challenges posed by innovative health technologies. The discussions underscored the importance of adapting HTA methodologies to assess emerging technologies like ATMPs, digital health, and high-cost treatments. There was a consensus that the HTA community is making progress but must continue to evolve to meet the complexities of these innovations. Key recommendations included (1) improving collaboration between HTA bodies and payers; (2) developing global frameworks for assessing new technologies; and (3) ensuring that the quality of data used in evaluations is robust. Cross-border collaboration and the sharing of best practices were seen as crucial to improving global decision making and ensuring equitable access to life-changing treatments.

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HEOR NEWS

1 Changing Life Expectancy in European Countries 1990-2021: A Subanalysis of Causes and Risk Factors From the Global Burden of Disease Study 2021 (The Lancet Public Health)

In assessing how changes in risk factors and cause-specific death rates in different European countries related to changes in life expectancy in those countries before and during the COVID-19 pandemic, researchers found that Norway, Iceland, Belgium, Denmark, and Sweden maintained improvements in life expectancy after 2011 through better maintenance of reductions in mortality from cardiovascular diseases and neoplasms, underpinned by decreased exposures to major risks. Read more

2 Excess Mortality and Years of Life Lost From 2020 to 2023 in France: A Cohort Study of the Overall Impact of the COVID-19 Pandemic on Mortality (BMJ Public Health)

The COVID-19 pandemic has had a long-lasting effect, with 4 consecutive years of excess mortality and a growing impact on people under 60, particularly men, suggesting lingering and profound disruption to the healthcare system. Read more

3 Cost-effectiveness of Pembrolizumab With Chemoradiotherapy for Locally Advanced Cervical Cancer (JAMA Network Open)

While adding concurrent and adjuvant pembrolizumab to first-line treatment with chemoradiotherapy and brachytherapy for patients with newly diagnosed, locally advanced cervical cancer significantly improved survival in these patients, at a willingness-to-pay threshold of $100,000 per quality-adjusted life-year (QALY), the addition is not cost-effective at current prices. Read more

4 Implementation Strategies of Financial Navigation and Its Effects on Alleviating Financial Toxicity Among Cancer Survivors: A Systematic Review (BMJ Quality and Safety)

Using the theoretical framework of implementation science, researchers determined that financial navigation is a potentially beneficial intervention for lessening the financial toxicity of cancer survivors, but more high-level evidence is needed for further validation. Read more

5 Institute for Clinical and Economic Review Publishes Special Report on COPD Therapies as Part of CMS Public Comment Process on Medicare Drug Price Negotiations (ICER)

According to ICER's analysis, Trelegy Ellipta (fluticasone furoate, umeclidinium, and vilanterol inhalation powder) and Breo Ellipta (fluticasone furoate and vilanterol inhalation powder), both once-daily inhalers for chronic obstructive pulmonary disease (COPD), have comparable or incremental net health benefits to generic therapies needing to be administered twice a day. Read more

6 Is Supported Employment Effective for Disability Insurance Recipients With Mental Health Conditions? Evidence From a Randomized Experiment in Belgium (Journal of Health Economics)

Compared to regular rehabilitation, Supported Employment increases the probability that Disability Insurance recipients with mental health conditions work while on claim, and there are significant effects for regular employment that is not publicly subsidized, since subsidized employment increases the cost of return-to-work programs, potentially during the whole career of the beneficiaries. Read more

7 Scaling Up Structured Lifestyle Interventions to Improve the Management of Cardiometabolic Diseases in Low-Income and Middle-Income Countries: A Systematic Review of Strategies, Methods and Outcomes (BMJ Public Health)

Dietary changes, physical activity, tobacco cessation, and alcohol intake are just some methods that have been proven effective at preventing and managing cardiometabolic disease, and the implementation of structured lifestyle interventions using different strategies such as engaging family and community to cocreate adaptations and early resource assessment can lead to improved outcomes of scale-up implementation. Read more

8 A Virtual Cardiometabolic Health Program Among African Immigrants in the USA Pilot Cluster-Randomized Clinical Trial (JAMA Open)

This pilot cluster-randomized clinical trial that included 60 African immigrants tested the effectiveness of a culturally adapted, virtual lifestyle intervention on control of blood pressure and hemoglobin A1c levels among African immigrants with cardiometabolic health risk factors. Participants in the study, who received a 6-month culturally adapted lifestyle intervention based on the National Diabetes Prevention Program (DPP) curriculum, delivered via virtual group sessions by a lifestyle coach of African origin, achieved reductions in systolic and diastolic measures. Read more

9 Primary Care Quality and Provider Disparities in China: A Standardized-Patient-Based Study (The Lancet Regional Health Western Pacific)

As the world's largest developing country, China has made significant investments in primary care over the past decade, but researchers documented a poor quality of primary care in China, with notable disparities across different providers. Urban community health centers provided relatively reliable primary care, and online platforms outpaced rural clinics, county hospitals, and migrant clinics in many areas, showcasing their potential to enhance access to quality healthcare resources in underresourced rural regions. Read more

10 NICE Joins International Collaboration on HTA Methods Research (NICE)

The UK's NICE has joined with the US-based Institute for Clinical and Economic Review (ICER) and the Canadian Drug Agency (CDA-AMC) to establish the Health Economics Methods Advisory (HEMA), a new international initiative to research and evaluate health technology assessment methods. HEMA's aim is to critically and independently research some of the most pressing topics in global health economics and health technology assessment methods. Read more

Section Editor: **Paula Lorgelly, PhD**, Aukland, New Zealand

Editor's Note:
Value & Outcomes Spotlight is pleased to introduce "HEOR Across the Globe" as a recurring section in the magazine. The Section Editors work with a small team of Regional Reporters from Asia Pacific, Latin America, and Eastern Europe, Middle East, and Africa to cover developments in health policies, news, and events from these regions.

Expectations Aplenty for New Zealand's HTA Agency

Paula Lorgelly, PhD, University of Auckland, Waipapa Taumata Rau, New Zealand

Aotearoa New Zealand's health technology assessment (HTA) agency, Pharmac, is unique as an HTA agency as it is both the decision maker and the funder of medicines. Their statutory objective is to secure the best health outcomes for eligible people in need of pharmaceuticals within the amount of funding provided. Like many agencies internationally, there has been criticism of the decisions it makes, particularly on how long it takes for medicines to be funded.

In late 2023, Aotearoa New Zealand held a general election in which Pharmac's functions and processes were featured in every main political party's manifesto. The proposals ranged from promises of ring-fenced funding for cancer drugs to replacing Pharmac with a new patient-focused medicines buying agency. Pharmac featured in both coalition agreements and the National-led coalition government created a new ministerial portfolio specifically for Pharmac. Most recently Pharmac received its instructions (or expectations) from the Minister regarding how it is expected to perform (indeed reform).

The Letter of Expectations lists 21 specific requests for Pharmac to consult on and ultimately take action. These include reviewing the dual role of value assessment and procedure, updating methods to include the wider fiscal impacts of funding or not funding medicines and the availability of tools to consider the wider societal impact, and considering how those with lived experience can be part of the decision-making process.

The Chair of Pharmac has accepted these expectations, and work is ongoing with both the board and the agency to understand the impact of these expectations. At the October 2024 Board meeting, Pharmac's dual role as decision maker and funder was discussed and the advice back to the Minister will be for these roles to remain colocated.

There is much interest in how Pharmac will address the expectation to consider the wider fiscal/societal impact. Currently, the perspective employed in economic evaluation evidence is that of the health system, the costs and cost savings for Pharmac, and the impact on primary and secondary care. There are several different ways to expand the evaluation space, and HTA agencies around the world differ in their scope. Previous National governments have promoted a social investment approach to understand the value and impact of interventions in the social services space. How this approach could be aligned with an economic evaluation using cost per quality-adjusted life year is yet unknown.

Watch this space! Will Aotearoa New Zealand offer up another unique HTA perspective? And will it address the long-held criticisms of the agency?

Health Insurance Reform in South Korea

Sang-Soo Lee, PhD, MBA, Medtronic North Asia (Korea and Japan), Seoul, South Korea

A forum was held on 9 January 2025 to discuss elements of South Korea's public and private health insurance reform. Reform is needed to address systemic inefficiencies, reduce financial burdens on citizens, and ensure sustainable healthcare delivery for all.

The National Health Insurance Service (NHIS), South Korea's single national payer, presented its proposals to improve the management of noncovered services. The primary goals are to ensure that essential treatments currently excluded are covered, while strengthening oversight of excessive or overused noncovered services.

Key measures include expanding coverage by way of transitioning essential treatments—such as surgical materials—into the scope of insurance coverage based on cost-effectiveness. NHIS is also looking to introduce managed coverage, a new category for noncovered services prone to overuse. This managed coverage will include new treatment guidelines and pricing criteria. Finally, NHIS is considering limiting coverage for nonmedically necessary services (eg, cosmetic surgeries) when performed alongside insured treatments. A final proposal for the national health insurance is to regularly reassess noncovered services to help eliminate treatments with questionable efficacy or safety, ensuring better resource allocation and patient care.

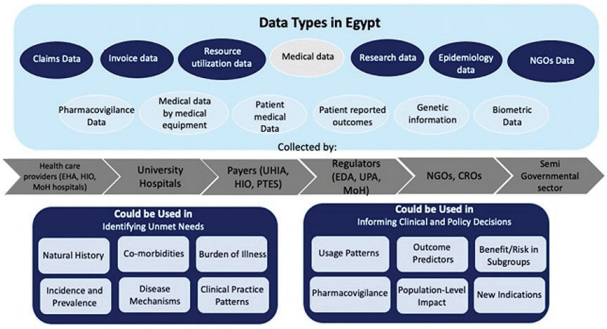
At the same forum, the Financial Services Commission (FSC) shared plans to reform private insurance with the aim of addressing medical overuse, correcting market imbalances, and reducing unnecessary utilization of minor noncovered services by a small percentage of users. The FSC is seeking to align copayment rates, focusing on severe illnesses (eg, cancer and cardiovascular diseases), while limiting coverage for overused noncovered services and improving private insurance disclosures to build trust and ensure fair practices.

The wider discussions at the forum emphasized the need to reallocate savings to protect essential medical services and restoring balance within the healthcare system. The next steps involve refining the proposals given the stakeholders' input from the forum, with detailed plans to be announced in the near future.

Data Classification in Egypt

Gihan Hamdy Elsisi, MSc, PhD, The American University in Cairo, Egypt

Egypt's healthcare system faces several current challenges regarding data classification. One major challenge is the lack of standardized data classification frameworks, which hinders the effective management and sharing of healthcare data across various parties.



CRO indicates Contract Research Organizations; EDA, Egyptian Drug Authority; EHA, Egypt Healthcare Authority; HIO, Health Insurance Organization; MOH, Ministry of Health; NGOs, Non-Governmental Organizations; PTES, Program of Treatment at the Expense of State; UHIA, Universal Health Insurance Authority; UPA, Egyptian Authority for Unified Procurement, Medical Supply, and Technology Management.

This figure shows the different public data types, uses, and its multiple sources (Egyptian Authority for Unified Procurement, Medical Supply, and Technology Management; Universal Health Insurance Authority; Egypt Healthcare Authority; Ministry of Health; Health Insurance Organization; Egyptian Drug Authority; Program of Treatment at the Expense of State; Non-Governmental Organizations; Contract Research Organizations).

Claims data, medical data, epidemiology data, and nongovernmental data could be used in identifying the unmet needs of disease natural history and comorbidities, incidence and prevalence of different diseases, clinical practice patterns, and disease mechanisms. These data could also be used in informing clinical and policy decisions in usage patterns, outcome prediction, benefits/risks in different subgroups, pharmacovigilance, and new indications.

Invoice data, research data, and resource utilization data could be used to identify the unmet needs of burden of diseases and also could be used in informing policy decisions in population-level impact and identifying the benefits/risks in different subgroups. It is important to classify data to be able to share it among different stakeholders and make informed decisions.

What Is the Cost of Vision Impairment and How Can We Reduce It? A Call to Action for Bulgaria

Maria Dimitrova, PhD, Alexander Oscar, PhD, Zdravko Kamenov, PhD, Maria Kamusheva, PhD, Konstantin Tachkov, PhD, Radka Goranova, PhD, Kostadin Angelov, PhD, Alexander Simidchiev, MD, Zornitsa Mitkova, PhD, Guenka Petrova, DSc, Medical University of Sofia, Bulgaria; **Petia Stratieva, MD, PhD**, Retina Bulgaria, Sofia, Bulgaria; **Rosen Dimitrov, MSc**, NRetia Health, Sofia, Bulgaria

Recent studies have shown that in Bulgaria, age-related macular degeneration constitutes a huge economic burden. Key factors contributing to the total economic burden are direct medical costs and productivity losses due to impaired or lost vision.

In 2024, an expert meeting organized by the Bulgarian Association of Pharmacoeconomics and the ISPOR Bulgarian Chapter discussed a strategic direction with a proposal for specific activities aimed at creating a National Eye Care Program and a National Prevention Center that would focus on the early detection of vision problems in both children and adults. The experts called for a comprehensive approach to eye health and making it a priority area. They agreed that implementing current recommendations for prevention (ie, screening and early diagnosis), as well as timely treatment of eye diseases according to modern standards, should start from early childhood.

This issue will be further discussed between the ISPOR Bulgaria, responsible institutions and key opinion leaders. Patient awareness programs will be organized to strengthen prophylactics.

First International Conference on Drug Pricing Policy in Serbia

Guenka Petrova, DSc, Medical University of Sofia, Bulgaria

A new international conference on drug pricing, The Influence of Pricing Policy on Drug Availability, will be held on 28 March 2025 in Belgrade, Serbia.

The conference gathered together representatives of various stakeholders from the European Union and Serbia, as well as from neighboring Adriatic countries. The main aim of the conference was to create a platform for discussion on the new regulations, share experience gathered in different countries, and to propose measures to improve patients' access to and affordability of medicines.

The topics of the conference covered hot issues related to medicine availability and affordability in Central and Eastern European Countries. The drug pricing policies in Montenegro, North Macedonia, Bosnia and Hercegovina, and Serbia were discussed in the first plenary session. The second plenary session was dedicated to economic factors influencing drug availability. The last session was devoted to cooperation between the countries in the region.

Colombian Healthcare System Faces Challenges Amid Reforms

Diego Rosselli, MD, EdM, MSc, Pontificia Universidad Javeriana, Bogotá, Colombia

Colombia's healthcare system, long considered a success story in Latin America, is experiencing significant changes that have raised concerns among medical professionals and patients alike.

The system, established in 1993, achieved universal coverage for the country's 50 million inhabitants and maintained low out-of-pocket expenses. It provided access to treatments for rare diseases, chronic conditions, and high-cost cancer drugs, often surpassing the capabilities of neighboring countries.

During the COVID-19 pandemic, the system demonstrated its strength by covering expensive treatments and achieving high vaccination rates. However, recent reforms have begun to alter the landscape of healthcare delivery in Colombia.

The core of the health system's financing, previously a combination of public and private insurers reimbursed through a capitation-based scheme, is undergoing modifications. The new approach aims to have health providers receive direct payments from the government with streamlined audit processes. These changes have led to uncertainty in the healthcare sector.

The immediate effects of these reforms are now becoming apparent. Several small- and medium-sized clinics have closed their doors, particularly impacting obstetrical and pediatric services. Patients are experiencing longer wait times for specialist appointments and surgical procedures. Some individuals requiring orphan drugs or chemotherapy have reported difficulties in accessing these treatments. Healthcare professionals are also feeling the impact of these changes, with many expressing worry about the system's ability to maintain the quality of care previously provided.

As Colombia navigates this period of transition in its healthcare system, the medical community emphasizes the importance of ensuring continued access to quality care for all citizens. The coming months will be crucial in determining the long-term effects of these reforms on the nation's healthcare landscape.

If you have ideas for a story or want to contribute an update, please email voseditor@ispor.org.

HTA POLICY UPDATE

Section Editors:
Sandra Nestler-Parr, PhD, MPhil, MSc; Ramiro E. Gilardino, MD, MSc

Welcome to the HTA Policy Update, which provides a brief update on notable HTA policy developments from around the globe. We welcome suggestions and guest editorials for future issues. Please contact the *Value & Outcomes Spotlight* editorial office with your ideas.

EU Joint Clinical Assessment

The European Union (EU) has advanced the implementation of the Health Technology Assessment Regulation (EU HTAR), effective since January 12, 2025. The regulation aims to streamline clinical evaluations of health technologies across EU member states, reducing duplication and facilitating the accelerating patient access to innovative treatments.

Under this framework, Joint Clinical Assessments (JCAs) are now mandatory for new oncology medicines and advanced therapy medicinal products submitted to the European Medicines Agency for marketing authorization. From 2028, all novel orphan drugs will be subject to EU HTAR, followed in 2030 by all other drugs, in vitro diagnostics, and high-risk medical devices. Early experience suggests a structured and harmonized approach of the JCA, with EU authorities collaborating with industry stakeholders.

The Belgian HTA body, INAMI-RIZIV, is to lead the EU consortium of 34 HTA bodies from 21 member states, following the January 2025 framework contract award. Drawing on its BeNeLuxA coalition experience, the consortium is backed by €35 million from the EU4Health Program and operates under the Heads of HTA Agencies Group and the HTA Coordination Group to enhance collaboration and transparency in JCAs and Joint Scientific Consultations (JSCs).

The European Commission launched the first submission window for JSCs on medicinal products from February 3 to March 3, 2025, with the next round set for June 2-30, 2025. The European Commission's implementing act for JSCs on medical devices, adopted on January 24, 2025, ensures transparency and consistency for medical devices and in vitro diagnostics.

Stakeholder engagement is prioritized, with ongoing workshops and guidance documents aiding navigation, and a stakeholder network meeting planned for July 2025.

As the regulation takes full effect, EU officials are committed to refining processes to improve efficiency and promote timely access to high-value health technologies.

International Collaboration

In a landmark collaboration, the Institute for Clinical and Economic Review (ICER) in the United States, the United Kingdom's National Institute for Health and Care Excellence (NICE), and Canada's Drug Agency (CDA-AMC) launched the Health Economics Methods Advisory (HEMA) initiative.

This initiative unites HTA experts, methodologists, and academics to advance research on global health economics and HTA methodologies.

Key objectives of HEMA include:

- Evaluating health economic methodologies to assess their applicability in HTA decision making
- Providing guidance and recommendations on the adoption and modification of novel methodologies
- Publishing research findings through white papers, peer-reviewed articles, workshops, and webinars to inform HTA best practices internationally

Chaired by Professor Mark Sculpher of the University of York, HEMA integrates diverse perspectives from methodologists, patients, and industry experts, aiming to generate evidence-based guidance adaptable to different HTA contexts. As healthcare decision making grows more complex, HEMA's work is expected to shape the future of HTA methodologies, supporting evidence-based policy across the United States, the United Kingdom, Canada, and other interested jurisdictions.

In March 2025, HEMA selected its first research topic, focusing on the benefits of treatment that should be considered in HTA decision making. With growing literature on additional benefits in economic analyses, HEMA will evaluate which proposed benefits—such as increased patient hope—should be incorporated into HTA studies. The project will develop specific guidance for HTA organizations on identifying new benefits, implementation strategies, and measurement challenges. The scoping phase is now underway, with a final report expected in December 2025.

Country Updates

Spain has refined its HTA framework with a new Royal Decree, enhancing transparency, efficiency, and patient involvement in the evaluation process.

Initially proposed by the Spanish Ministry of Health in August 2024, the decree establishes a structured and independent HTA system aligned with the EU HTAR. A key feature is the distinction between clinical evaluations, assessing the added clinical value of health technologies and non-clinical evaluations, which consider cost-effectiveness, ethical implications, and social impact.

The governance structure includes:

- Governance Council, chaired by the Secretary of State for Health, with health economists, patient representatives, and regional authorities
- HTA Evaluation Offices under the Spanish Agency of Medicines and Medical Devices (AEMPS), responsible for clinical and economic assessments
- Health Technology Appraisal Group, synthesizing findings to guide National Health System (SNS) decisions

The decree emphasizes real-world evidence and patient input and, after a period of consultations, the final version is now under review by the State Secretariat Council, with adoption and enactment expected by April 2025.

In addition, Spain's Pharmaceutical Industry Strategy 2024-2028, launched in December 2024, aims to align HTA, pricing, and reimbursement systems while boosting research and development and real-world evidence generation. The plan introduces a new cost-effectiveness evaluation system, early dialogue with authorities, accelerated access pathways, and public-private partnerships to drive innovation. These measures position Spain to adapt to evolving EU regulations and aim to enhance patient access to medicines.

Emerging Trends To Watch

The German Institute for Quality and Efficiency in Health Care (IQWiG) is refining its General Methods (Version 8.0) to better integrate patient perspectives in HTA. Through questionnaires, interviews, public consultations, and committee representation, IQWiG aims to gather input from patients with lived experiences. Continuous evaluation and support for plain language summaries will enhance transparency and accessibility. Stakeholders can submit comments on the draft until April 29, 2025.

The French National Authority for Health (HAS) unveiled its 2025-2030 strategy, emphasizing economic evaluations to better assess the efficiency of innovative health technologies. With the establishment of a new economic evaluation department, HAS aims to manage uncertainty in benefit assumptions, strengthen early dialogue with innovators, and enhance public decision-making support.

The editors acknowledge this issue's contributions from Dr. Dan Ollendorf, Dr. Jose Diaz, and Dr. Carlos Martin Saborido.

METHODS EXPLAINED

In this edition of Methods Explained we are covering discrete event simulation (DES) based on a conversation with Hendrik (Erik) Koffijberg, PhD. He is professor of Technology Assessment of Digital Health Innovations and chair of the section Health Technology & Services Research at the University of Twente in The Netherlands, where he teaches several courses on health economic modeling. He is also a member of the scientific advisory board of the National Healthcare Institute. Together with other experts, Erik and I have published several methodological and practical papers on DES, and we continue to teach postgraduate courses and short courses on the topic.

Discrete Event Simulation

Section Editor: **Koen Degeling, PhD**

What is DES and what can it be used for?

DES is a simulation technique that originates from operations research. It dates back to the 1950s and has been used extensively to investigate and optimize logistic, manufacturing, and operational processes that are characterized by scarce resources and queues.

In line with its origin, the first applications of DES in healthcare focused on optimizing care processes on an operational level, such as in hospitals. Examples include optimizing emergency department layout and staffing, as well as operating room scheduling, which are settings characterized by limited resources and variable demand.

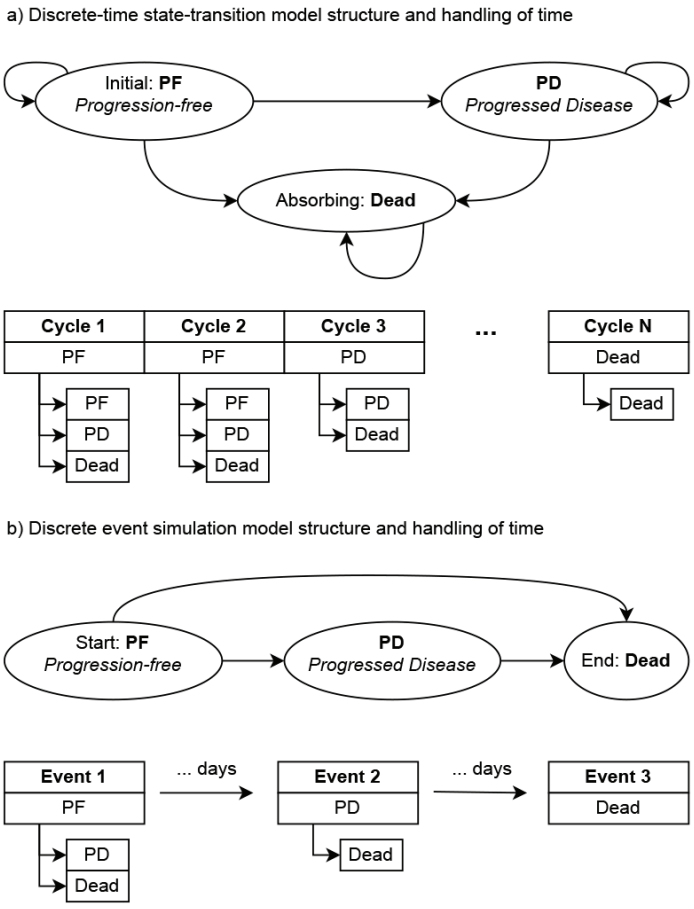
DES has also proven valuable as a decision-analytic modeling technique within the field of health economics and outcomes research (HEOR). Like decision trees, Markov models and partitioned-survival models, it can be used to perform model-based economic analyses, such as cost-effectiveness analyses. It is also well suited to model the (re)design or optimization of health services and support implementation decisions.

How does DES work and what makes it different from similar methods?

To understand how DES works, it is useful to compare it to other well-known and commonly used cohort-level modeling methods, such as partitioned-survival modeling and Markov modeling (ie, cohort-level state-transition modeling), and to other individual-level modeling methods (ie, microsimulation), such as individual-level state-transition modeling.

DES is an individual-level modeling method like individual-level state-transition modeling, which implies that it simulates hypothetical individuals or "entities," each with their own characteristics and history. This is different from cohort-level methods, such as Markov modeling and partitioned-survival modeling, which model an average cohort by keeping track of proportions of the cohort assigned to certain health states over time. By simulating individuals, DES is able to account for differences in events and outcomes between individuals that can be explained (ie, heterogeneity) and that arise due to random chance (ie, stochastic or first-order uncertainty).

Figure 1. Illustration of the model structure and handling of time for the commonly used 3-state oncology model for: (a) a discrete-time state-transition model (eg, Markov model) and (b) a discrete event simulation model.



Something that really sets DES apart is its handling of time. Most state-transition models are discrete-time methods, which means that they deal with time in fixed (discrete) steps by repeatedly applying state transitions based on probabilities. This is illustrated in the hypothetical example in **Figure 1**, where

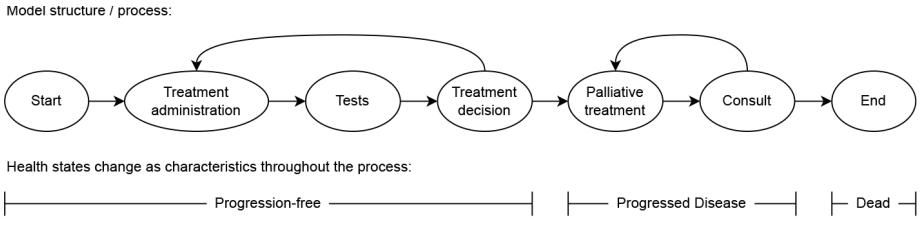
transitions to different (or the same) health states are applied repeatedly. On the other hand, DES is a continuous-time method and it jumps from event to event, which can occur at any point in time. The impact of this difference is that DES is more efficient and potentially more accurate compared to discrete-time methods. Furthermore, this makes it straightforward to implement parametric and empirical time-to-event distributions in a DES, with parametric distributions being commonly used in HEOR to extrapolate observed survival data.

Another difference between DES and state-transition models is that the latter are typically structured based on health states, whereas DES models are often specified based on more granular events or processes where the health state is defined as a characteristic of the simulated individuals. **Figure 2** presents a model structure that resembles the same clinical pathway as **Figure 1** but is structured based on more granular events that can be mapped directly to the impact of those events, such as the cost and impact on quality of life of starting a new treatment. This decoupling of the model structure and health states may allow for a more straightforward and realistic translation of clinical pathways into model structures. For example, this makes it possible to model diagnostic tests without those necessarily impacting the health state of the individual. It also allows more detailed and transparent definition of costs and health effects in the model, by linking these to the granular events in the model structure.

Finally, its ability to readily model resources and capacity constraints, and how simulated individuals interact with each other through those resources, are unique to DES. Examples of resources in a healthcare setting include machinery, such as certain hospital or laboratory equipment, but certainly also the time of healthcare providers. Queues and waiting times, and their impact on relevant outcomes, can easily be simulated by specifying required and available resources. Although interaction between entities of the same type (eg, patients) can theoretically be modeled using DES, this is highly complex due to its continuous handling of time and, hence, this is typically done using agent-based models (a discrete-time individual-level modeling method).

Overall, compared to more traditional modeling techniques used in HEOR, DES offers increased flexibility to represent complex clinical pathways without simplifying assumptions and facilitates consideration of resources and capacity constraints. Therefore, DES would be the preferred technique when explicitly modeling resources, whereas for typical health economic models, the added value of DES increases with increasing complexity of the modeled pathway and larger amounts of information being available to populate the model.

Figure 2. Illustration of how the commonly used three-state oncology model of Figure 1 could also be implemented as a care process based on more detailed events and with health states modeled as characteristic for each of the simulated individuals.



What are the steps in using DES and what software is available?

Fundamentally, there are no differences in the steps involved in applying DES compared with individual-level state-transition modeling. What makes these microsimulation methods different from the cohort-level techniques is the simulation of the population of hypothetical individuals with their (correlated) characteristics and the need to assess whether sufficient individuals are simulated to obtain stable outcome estimates.

In terms of software, DES has traditionally been available through proprietary software, such as Simul8, AnyLogic, Arena, and TreeAge. Like state-transition models, relatively simple DES models that do not consider resources can be implemented in Excel, but such applications seem to be rare. As an alternative, freely available code-based environments like R and Python have been used increasingly for DES, with R being most prominently used within HEOR. Although proprietary software generally provides convenient user interfaces, they can be costly and may not necessarily be as transparent, flexible, and reusable as code-based alternatives. With initiatives like the R for Health Technology Assessment (HTA) consortium, the use of code-based environments to implement DES models within HEOR may increase further.

What is the current level of adoption of DES in HEOR?

DES has been used widely in research and organizations across various disciplines, including healthcare organizations, where it has been used to inform decisions on the organization of care on national, regional, and local levels. It is increasingly used in HEOR and there are plenty of DES applications in academic HEOR projects. Although it is accepted as a modeling technique by most HTA agencies, use in reimbursement submissions has been relatively rare.

The use of DES in HTA submissions may increase over the coming years as clinical pathways continue to become more complex. There may also be more applications due to an increasing interest in considering workforce capacity and environmental sustainability as part of HTA (eg, at the National Healthcare Institute in The Netherlands). Increased use of advanced evidence synthesis methods to analyze and combine information from various sources, such as multilevel network-meta regression that was covered in a previous edition of Methods Explained, may also contribute to the further adoption of DES. Individual-level techniques like DES can fully utilize the output of such analyses, whereas cohort-level techniques require such outputs be aggregated to the average cohort being modeled, resulting in a loss of information.

What are the remaining challenges in the uptake of DES?

There are several perceived challenges regarding the use of DES. The computational burden of individual-level modeling

techniques in general was a barrier to their use previously, but nowadays these models can be easily run on a standard laptop. Potentially the biggest perceived challenge is that individual-level models, or DES specifically, need more data compared to cohort-level state-transition models. This is not true. If there is scant but sufficient evidence to develop a Markov model, the same evidence can be used to develop a DES model. The added value of DES in that scenario may be limited, although it may still facilitate a straightforward integration of the evidence and more accurate representation of clinical practice, especially for complex pathways. More detailed evidence becoming available at a later stage would also be more easily implemented in a DES model, through more granular events.

As most HEOR professionals are trained in using state-transition models, the steep learning curve is a real barrier to using DES. For example, it is typically required to write at least some code to implement a DES. The most challenging aspect, however, turns out to be the model conceptualization. Because DES offers a lot of flexibility, there are often multiple ways to translate a clinical pathway full of events of varying importance into a model structure. With increasing familiarity through methodological guidance, examples, tutorials and (short) courses, as well as a new generation of modelers being trained in code-based software like R, the barriers to leveraging the power of DES will likely continue to decrease.

What are some key references for further reading?

A good starting point for reading more about DES in the context of HEOR is the ISPOR-SMDM Task Force report.[1] However, given that substantial progress has been made in adapting the DES methodology for HEOR since its publication, this report from 2012 is not fully up to date anymore. Throughout 2025, a tutorial and book chapter on the implementation of health economic DES models in R are expected, but these have not been published yet.

In terms of examples, a review by Vázquez-Serrano et al presents various operational research studies leveraging DES in healthcare.[2] With regard to HEOR, one of my own publications showcases the value of DES for modeling the complex treatment pathway of prostate cancer based on only aggregate data from literature.[3] The work by Blommestein et al illustrates how real-world data can be combined in a DES model with effect estimates from network meta-analyses to model treatment sequences in multiple myeloma.[4]

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We welcome feedback and suggestions for topics or experts in the field for future issues. Please contact the *Value & Outcomes Spotlight* editorial office at voseditor@ispor.org with your ideas.

UNLOCKING AFFORDABILITY & ACCESS WITH HEOR

BY BETH FAND INCOLLINGO

Healthcare can only be widely effective if most patients can access and afford it. Yet, even within the most successful delivery systems, a host of barriers can interfere with health equity.

In high-income countries, the challenge is to provide comprehensive healthcare while preventing its overutilization. Countries with universal healthcare achieve that by providing only the drugs and services expected to be cost-effective, which can lead to out-of-pocket spending for patients who want more. The United States takes different tacks, one of which is to limit overuse through patient cost-sharing. Unfortunately, that often stresses household budgets and prevents people from obtaining needed care.

Meanwhile, many low- and middle-income countries (LMICs) grapple with limited market access to essential drugs, often coupled with insufficient mechanisms for reimbursing patients' spending.

A 2023 report by the World Bank and the World Health Organization (WHO)[1] found that at least half the world's population—4.5 billion in 2021—lacks access to essential health services, with many experiencing poverty due to healthcare costs.

"The fact that so many people cannot benefit from affordable, quality, essential health services not only puts their own health at risk, it also puts the stability of communities, societies and economies at risk," said WHO Director General Tedros Adhanom Ghebreyesus, PhD, MSc in an article published by the organization's Pan American Health Organization.[2]

Experts conducting health economics and outcomes research (HEOR) are working to boost healthcare's accessibility and affordability by identifying cost-effective ways to support individual access and worldwide economies and health.

"Fundamentally, HEOR helps us understand what we get for what we pay and whether spending money in a certain area provides us with worthwhile benefit," said Anna Sinaiko, PhD, a health economist with Harvard's TH Chan School of Public Health. "We have limited resources available in our healthcare system and our society, and we want to know what we're getting in terms of outcomes related not only to health, but to patient experience, satisfaction, and quality of life."

Strengthening Universal Healthcare

Reinhard Busse, Dr. med., MPH, head of the Department of Health Care Management in the Faculty of Economics and Management at the Berlin University of Technology, believes that universal healthcare—which citizens typically support through a flat-rate tax or a fixed percentage of their income—is the best way to ensure widespread and affordable access to essential services. Public systems should offer comprehensive, quality care to all or most, he said, with cost sharing limited to prevent financial hardship.

For instance, in Germany, cost sharing stops after patients have spent 2% of their gross incomes on healthcare—or 1% if they are chronically ill.[3] That's a much lower limit than in the United States, where the poorest residents spend 35% of their pretax incomes on healthcare while those in the country's highest income decile lay out 3.5% of their pretax dollars.[4]

Successful public systems should also provide care opportunities that are geographically within reach for patients, with a wide range of facility hours and reasonable appointment wait times, he said, adding that patients should be able to choose doctors who speak their language and match their preferences regarding age, gender, ethnicity, or religion.

Cost challenges can include physician recommendations for care that exceeds the covered basics. For instance, patients may decide to pay out of pocket if their dentists recommend gold fillings instead of standard ones, while the system can be overburdened if orthopedic surgeons routinely prescribe unnecessary knee replacements.

"Politicians usually think the solution is to delist services," Busse said, "but it's really more about using HEOR to find the right target group for an intervention and making sure that only those people get it."

"The fact that so many people cannot benefit from affordable, quality, essential health services not only puts their own health at risk, it also puts the stability of communities, societies and economies at risk."

– General Tedros Adhanom Ghebreyesus, PhD, MSc

Busse noted that drug costs are the major driver of catastrophic spending for patients who live in countries with universal healthcare. According to a 2023 report by WHO's European Region,[5] the biggest cause is insufficient primary care coverage including drugs, which was identified in all 40 countries studied and largely affected the poorest 5th of households.

In addition to covering primary care more comprehensively, the report's authors suggested that countries limit copayments for people who are poor or sick, spend adequately, and cover everyone, including citizens who have fallen behind on their health insurance contributions and undocumented immigrants.

Inequities can also arise when voluntary private insurance is available alongside universal healthcare, Busse noted, as this may improve access and choice for the healthy and wealthy while drawing resources away from the public system.

Policy makers must consider cultural trends, too, he said, such as the common misconception that more healthcare is always better.

"In Germany, we have 50% more hospitalizations than the countries surrounding us,"[6] Busse said, "which amounts to an overprovision of services."

Cultural preferences may also affect a country's medical priorities, he said.

"People in East Asia, especially in Japan, tend to hate surgery but love imaging," Busse said, "while in Germany, we believe that, if a surgeon has taken care of something, it has really been resolved."

Establishing Price Transparency in the United States

In the United States, cost sharing is designed to discourage unnecessary medical visits, but it often creates barriers to essential care, especially for those in lower income brackets, said Sinaiko, who studies how consumer decision making within the context of American healthcare policy can help drive cost-efficiency.

Due to extremely high prices for care and coverage in the United States, she said, many are uninsured or underinsured, creating health disparities for Black and Hispanic or Latino Americans compared with their White and Asian neighbors[7] and driving up overall healthcare spending. This creates a vicious cycle of ever-rising insurance premiums and increasingly limited access, she said.

> "Fundamentally, HEOR helps us understand what we get for what we pay and whether spending money in a certain area provides us with worthwhile benefit."
>
> – Anna Sinaiko, PhD

A survey conducted by the Commonwealth Fund[8] found that, in 2024, 9% of American adults were uninsured and 23% were underinsured. In addition, 12% had experienced a gap in coverage over the past year.

These inequities are perpetuated by the fact that various insurance types reimburse physicians at different negotiated rates for the same services, with private plans paying more than the country's public programs, Medicare and Medicaid.[9] Because Medicaid—which covers the poorest Americans—pays the least, Sinaiko said, the fewest providers accept it, deepening disparities in access.

Similarly, she said, patients pay different amounts for procedures and medications based on their insurance carriers and plans, even within the same hospitals and drug stores.

Because Americans don't usually have easy access to data about which facilities will provide them with care at the best value, they can have a hard time making thoughtful financial decisions, and that's why Sinaiko is exploring price transparency solutions.

Already in use are tiered insurance-plan networks that steer patients to their most cost-effective care options; self-insured employers that identify medical centers of excellence for their staff members based on value and quality; and price transparency tools offered by health insurers that estimate procedure costs at various facilities based on the parameters of each patient's plan.[10]

A recent executive order by President Donald Trump[11] could bolster compliance with efforts launched during his first term to increase price transparency in healthcare, which called for hospitals to reveal what they're paid for specific procedures and insurers to publish how much they disburse.

Also promising, Sinaiko said, is a Real-time Prescription Benefit Tool designed to help doctors identify the most affordable medications for individual patients.[12] Sinaiko's team is working with an early adopter of the health informatics tool, the University of Colorado Health System, to explore its potential. Implementation of such a tool will be required by the Centers for Medicare & Medicaid Services (CMS) by the start of 2027.[13]

The overall goal of these efforts, Sinaiko said, is to slow the growth of healthcare costs in the United States, and an additional way to achieve that is through government negotiations on drug prices—a strategy CMS has begun pilot testing under the Inflation Reduction Act.[14]

"The question going forward is whether any of these market interventions can put enough downward pressure on prices to meaningfully improve affordability," Sinaiko said. "If not, the government and other payers will likely move toward regulation to achieve this goal, such as by negotiating more drug prices or setting ceilings for commercial prices."

Boosting Access in LMICs

Due to healthcare inaccessibility, many diseases have lower survival rates and patient outcomes in LMICs than in high-income countries, said Moy Bracken, MPharm, a research unit manager with the Access to Medicine Foundation in Amsterdam.

For example, Bracken said, the survival rate for childhood cancers is approximately 80% in high-income countries but as low as 30% in some LMICs.[15] She added that tuberculosis, a disease largely eradicated via antibiotics in most high-income countries, remains the biggest global killer among all infectious diseases, having taken the lives of 1.25 million people in 2023 alone.[16]

These statistics are not surprising, Bracken said, as people in LMICs face a host of healthcare barriers, including:

- Government health systems with small budgets and little negotiating power
- Insufficient or absent public health reimbursement mechanisms, resulting in patients either paying out of pocket or foregoing crucial medications
- Shortages of qualified healthcare workers and clinics
- Limited access to diagnostic care
- Lack of access to drugs due to gaps in manufacturing, the supply chain and research and development, which often does not focus on therapeutics for the diseases that disproportionately impact LMICs
- Sociocultural barriers arising from inefficient patterns of help-seeking behavior and health literacy[17]

The independent, nonprofit Access to Medicine Foundation is doing its part by encouraging the pharmaceutical industry to provide essential resources in LMICs.

"Without the manufacturers and patent holders of lifesaving medications on board, it would be much more challenging to bridge those gaps in access," Bracken said. "Many international strategies for addressing major public-health problems in LMICs, such as malaria, tuberculosis, HIV, and antimicrobial resistance, are funded by international donors—both public and private—and one of their key partners is the pharmaceutical industry."

The foundation's initiatives include its Access to Medicine Index, which publicly ranks the efforts of 20 large research-based pharmaceutical companies to improve access to medications, vaccines, and diagnostics in LMICs. Most recently, the foundation launched research programs evaluating companies that manufacture and distribute generic drugs and those that supply medical oxygen.

"We evaluate companies' performance to identify gaps in access and engage with the industry to share best practices," Bracken said. "We also engage with investors committed to health equity who can drive change by bringing these issues into their conversations with companies."[18]

Companies can engage in inclusive business models to ensure sustainable access for their products in underserved populations.[19] Additionally, companies can work with product development partners, such as the Drugs for Neglected Diseases Initiative, to develop new therapies for diseases that disproportionately affect people in LMICs.

Pharmaceutical companies can also contribute by teaching healthcare professionals in LMICs to run clinical trials[20] and by providing products or care through memoranda of agreement with governments in those countries, Bracken said. And they can work with intermediaries such as the Medicines Patent Pool in Switzerland to offer licensing agreements that enable generic manufacturers to make versions of their brand-name drugs for use in LMICs.[21]

The latter mechanism has been used by companies such as Gilead[22] and GSK[23] to increase access to medicines for communicable diseases, including treatments for HIV and hepatitis C.

Companies including GSK[24] and MSD[25] also participate in pooled procurement mechanisms, engaging with the international entity Gavi, the Vaccine Alliance, to increase the affordable and high-quality supply of HPV vaccines and other inoculations for children in LMICs, and Bayer partners with the United Nations Population Fund to provide affordable access to female contraception.[26]

These initiatives can boost a company's reputation, but they can also create opportunities to generate profits, Bracken said, particularly in middle-income countries that have significant manufacturing capabilities, larger health budgets, and large populations.

> "Politicians usually think the solution is to delist services, but it's really more about using HEOR to find the right target group for an intervention and making sure that only those people get it."
>
> – Reinhard Busse, Dr. med. MPH

"Companies can implement tiered pricing models, where the price of the drug is determined by the country's income level, which means that countries with lesser ability to pay can procure drugs at a lower price," she said. "There is evidence to show that manufacturing locally can lower the cost of medicines, increase their availability, reduce taxes, and promote local sustainability."

Looking ahead, the foundation wants to better support LMIC access to treatments for noncommunicable diseases like cancer, especially as more precision medicines emerge that are expensive and require genetic testing for patient eligibility.

Addressing Global Concerns

Healthcare access and affordability depend upon international partnerships, and many of the world's governments are involved in that effort.

In 2022, Luxembourg led the way by donating a higher percentage of its national income than any other country—.46%.[27] Still, in terms of sheer dollars, the United States has far outstripped its counterparts; in 2023, it gave $9.5 billion in aid.[28]

Yet, a recent decision by the United States to halt much of its foreign aid threatens to affect healthcare access for 9 million

people in Afghanistan, 750,000 in Haiti, and numerous others who previously benefited from America's support of a global measles and rubella lab network and programs that curb devastation from tuberculosis and HIV.[29]

In making decisions about foreign aid, Bracken said, it's worthwhile for governments to consider the benefits of supporting healthcare in LMICs.

She noted that the United States significantly boosted its own economy by investing in American companies and institutions that conducted global health research and development.[30] In addition, she said, every country can benefit from efforts that keep infectious diseases from spreading.

> "LMICs comprise 80% of the world's population, so stakeholders have an ethical and moral responsibility to make essential medicines available there."
>
> – Moy Bracken, MPharm

"One example is dengue fever, which is spread by mosquitoes," Bracken said. "Traditionally, it's been prevalent in tropical countries, but now we're seeing cases in southern Europe and the United States because of climate change.[31] To prevent its spread requires international collaboration and funding mechanisms."

Ultimately, she said, policy makers need to consider their obligations as global citizens.

"LMICs comprise 80% of the world's population," Bracken said, "so stakeholders have an ethical and moral responsibility to make essential medicines available there."

HEOR is likely to factor into the bulk of decisions about access and affordability across a wide range of stakeholders, both domestically and internationally, she added.

"International procurers use HEOR to identify the most cost-effective solutions for different public-health priorities, and public payers and policy makers use it to determine the societal value of new innovations and efforts like HIV interventions," Bracken said. "HEOR can also help advocacy groups build campaigns that affect pharmaceutical-company priorities and pricing decisions, with the potential to broaden overall access and affordability."

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By the Numbers: Affordability and Access

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Assessing Implementation of User Fees and Their Impact on Access to Health Services in Low- and Middle-Income Countries in Africa

INTRODUCING user fees in countries **without** user fees resulted in:

- Decreased **-15.4%** immediately
- Decreased **-28%** to **-51%** immediately
- Decreased **-17%** after 12 months
- Changed **-9%** to **+8%** after 12 months

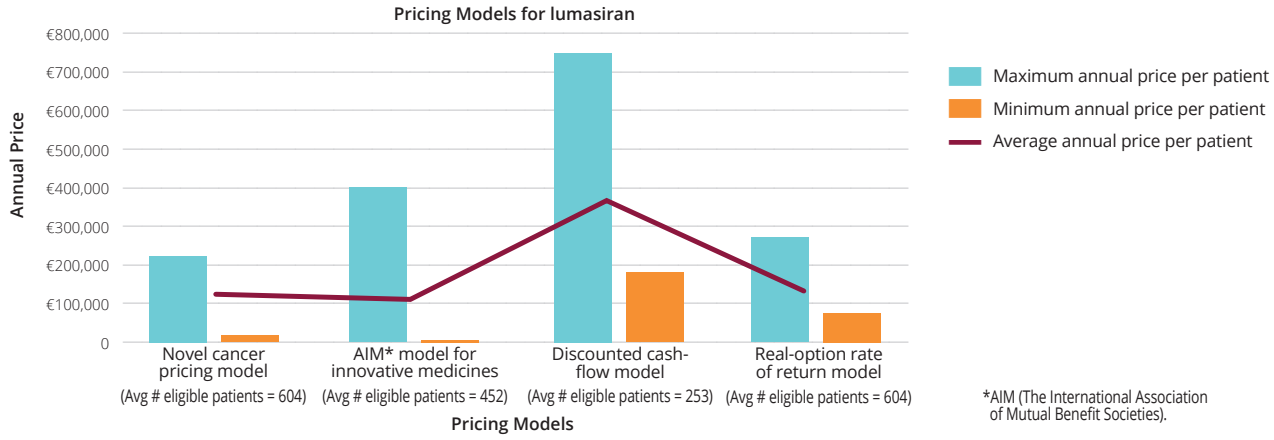
Utilization of **preventive** care | Utilization of **curative** care

REMOVING user fees in countries previously **with** user fees resulted in:

- Increased **+1.3%** to **+249%** immediately
- Increased **+30%** to **+50%** immediately
- Increased **+5%** to **+92%** after 12 months
- Increased **+18%** to **+93%** after 12 months

Utilization of **preventive** care | Utilization of **curative** care

Variation in Drug Price With Different Pricing Models for an Orphan Medication in Europe



How Biopharma Can Make a Difference in Health Equity

Progress made by biopharma companies on health equity

- **>50%** of companies **have formal plans** to address health inequities and include health equity goals in their strategies
- **>70%** of products **are covered** by at least 1 access strategy in LMICs
- **75%** of companies **track some metrics** related to health equity

The impact of this progress has not materialized

- **~40%** of the population in LMICs **does not have coverage** for essential health services
- **~6x** more likely for a product to be **registered in upper–middle-income countries** than low-income countries
- **>70%** of internationally recognized research and development priorities for LMICs **remain unaddressed**

Individual Reimbursement Approval Under a Managed Access Protocol for Liraglutide (Saxenda®) for Weight Management in Ireland: Design, Implementation, and Outcomes

Claire Gorry, PhD; Stephen Doran, MSc; Rosealeen Barrett, MSc; Michael Barry, MD, Health Service Executive Medicines Management Programme, Dublin, Ireland

Liraglutide (Saxenda®) for weight management is available through the publicly funded drugs schemes in Ireland, subject to a managed access protocol.

Reimbursement is supported for people with a body mass index ≥ 35 kg/m^2, prediabetes, and high risk for cardiovascular disease.

Managed access protocols support evidence-based and cost-effective use of medicines; enable patient access to innovative medicines; and provide oversight, governance, and budgetary certainty to the healthcare payer.

Introduction

The authorization of glucagon-like peptide-1 (GLP-1) analogues for weight management has led to a step-change in the treatment of obesity. While these treatments offer promise in terms of improving long-term morbidity and mortality outcomes for patients with obesity, there are significant challenges to their widespread adoption, including uncertainty regarding the optimal duration of treatment, questions around the high cost and affordability of these agents, the ability of manufacturers to provide sufficient product to meet demand, and attitudes regarding obesity as a lifestyle issue rather than a medical condition.[1] The high prevalence of obesity and the potential budget impact of widespread adoption of these agents represents a significant financial challenge to publicly funded health systems.[2]

In Ireland, the Health Service Executive (HSE) has adopted a novel approach utilizing a reimbursement application system (RAS) and a managed access protocol (MAP) to enable access to medicines for weight management in a cost-effective manner. Liraglutide (Saxenda®) is a GLP-1 analogue, and it is licensed as an adjunct to a reduced-calorie diet and increased physical activity for weight management in adult patients, with an initial body mass index (BMI) ≥ 30 kg/m^2, or BMI ≥ 27 kg/m^2 in the presence of at least one weight-related comorbidity such as dysglycemia, hypertension, dyslipidemia, or obstructive sleep apnea.[3] Liraglutide is reimbursed in Ireland by the HSE in a subpopulation of the licensed population, where there is a higher probability of cost-effectiveness, as described below.

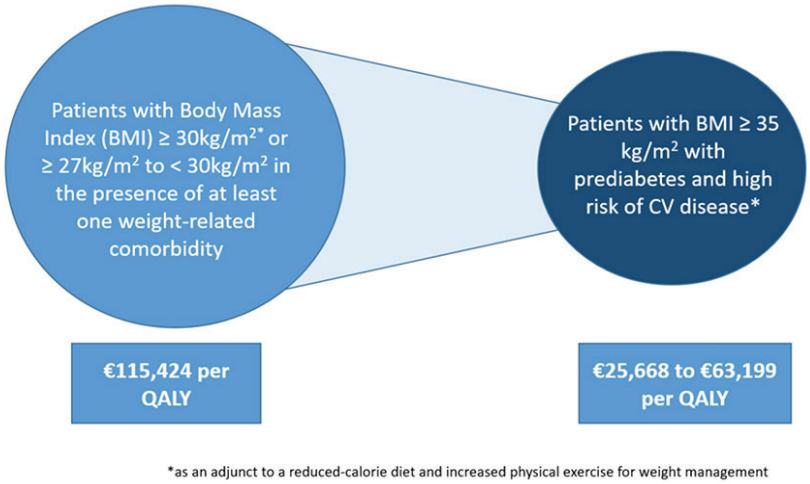
An RAS requires the prescriber to submit an individual patient application for reimbursement approval for a medicine. For liraglutide, this is done through an online portal, which is linked to the national pharmacy claims reimbursement system. Applications are reviewed within 3 working days of submission, and for patients who meet the eligibility criteria and are approved, this approval is visible to the prescriber in real-time following a review by a pharmacist member of the HSE-Medicines Management Programme (MMP) team. It is then also immediately visible to the community pharmacist dispensing the medication.

> The high prevalence of obesity and the potential budget impact of widespread adoption of these agents represents a significant financial challenge to publicly funded health systems.

A MAP lists the eligibility criteria attached to reimbursement support for a medicine. In order for an application submitted through the RAS to be approved, the patient must satisfy all of the eligibility criteria as outlined in the MAP. These criteria are developed based on a number of inputs, including the recommendations of the HSE Drugs Group regarding reimbursement conditions, commercial terms as negotiated with the marketing authorization holder (MAH), the product license, pivotal trials, and the health technology assessment (HTA) of the medicine, conducted as part of the pricing and reimbursement process. In this way, a MAP enables evidence-based and cost-effective utilization of medicines. It also provides the decision maker with a greater degree of clinical governance over the use of a medicine, and greater certainty regarding the utilization and expenditure of a medicine post-reimbursement.

The MAP for liraglutide was developed by the HSE-MMP.[4] The HSE Drugs Group recommended reimbursement support for a subpopulation of the licensed population (**Figure 1**), based on the population assessed in the HTA submission provided by the MAH.[5,6] This

Figure 1. Outcomes of the cost-effectiveness analysis of liraglutide in Ireland in the full licensed population (left), and the subgroup where reimbursement was recommended by the HSE Drugs Group (right).[5,6]



BMI: Body mass index; CV: cardiovascular risk; QALY: Quality-adjusted life year.

population consists of patients with a BMI ≥ 35 kg/m², with prediabetes and high risk of cardiovascular disease, where there is a greater probability of cost-effectiveness (**Figure 1**). The HSE Drugs Group also recommended that the MAP incorporate guidance on treatment discontinuation for patients who have not lost ≥ 5% of their initial body weight after 12 weeks of treatment with liraglutide 3 mg daily (ie, the maintenance dose), as per the product license.[3] This is operationalized via a two-phase approval process, outlined below. Reimbursement is supported for 2 years' duration, in line with the assumptions in the budget impact model submitted by the MAH.

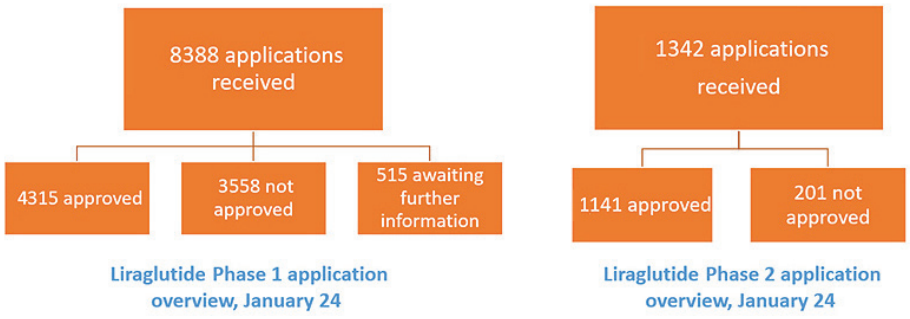
To be eligible for phase 1 approval, which provides reimbursement support for 24 weeks (6 months), patients must be aged between 18 and 74 years at time of application. Prescribers must confirm that the patient is actively participating in nonpharmacological interventions for weight management. Patients must have an established diagnosis of nondiabetic hyperglycemia (prediabetes), defined as a fasting plasma glucose of 5.5-6.9 mmol/L and hemoglobin A1c (HbA1c) level of 42-47 mmol/mol, based on readings taken within 30 days of the application. Patients must also be at high risk for cardiovascular disease, defined as fasting total cholesterol > 5 mmol/L or mean systolic blood pressure > 140 mmHg confirmed on a 24-hour blood pressure monitor. For applications where the cardiovascular parameters are outside of those specified, pharmacological management of same is taken into consideration.

There are a number of clinical exclusion criteria for reimbursement support, aligned with the criteria implemented in the SCALE Obesity and Prediabetes trial (NCT01272219) and the Summary of Product Characteristics.[3] These include people who are currently pregnant or breastfeeding, have developed obesity secondary to endocrinological or eating disorders, and those with uncontrolled hypothyroidism/hyperthyroidism. To be eligible for phase 2 approval, the prescriber must submit a phase 2 application providing the patient weight following 12 weeks of treatment with the maintenance dose of liraglutide (3 mg daily). The IT system will automatically determine if this is a weight loss of ≥ 5% compared with the baseline weight and will automatically apply reimbursement approval for responding patients, for up to a maximum of 2 years, treatment duration.

We presented an overview of applications received in the first 4 months of availability of liraglutide in Ireland at ISPOR Europe in November 2023.[7] We reviewed the 3081 phase 1 applications for reimbursement support submitted via the online reimbursement application system between January 1 and April 30, 2023. The majority of these applications were for females, with an average age of 50.6 years; 43.5 kg/m² was the average BMI. High-risk for cardiovascular disease was reported in 83.4% of the applications, and a confirmed diagnosis of prediabetes was reported in 48.2% of the applications. Reimbursement was approved for 1575 (53.4%) applications, as these patients satisfied all the criteria outlined in the MAP.

As of January 31, 2024, 8388 phase 1 applications for reimbursement support for liraglutide for weight management had been received, with 4315 patients approved for reimbursement support (**Figure 2**). Clinicians can submit applications on behalf of any patient; we have not formally analyzed the reasons for the high level of applications submitted on behalf of patients who do not meet the reimbursement criteria. Under phase 2, 1342 applications for reimbursement support had been received, with 1141 patients approved. There is a notable difference in the approval rate between the phase 1 and phase 2 applications; it seems likely that clinicians are mainly only submitting phase 2 applications on behalf of

Figure 2. Overview of applications received via the online reimbursement application system for liraglutide for weight management between January 1, 2023 and January 31, 2024.



patients who have attained the required weight loss within the time frame.

In January 2024, 1948 patients were accessing liraglutide under the Community Drug Schemes in Ireland. Cumulative expenditure on liraglutide in this timeframe was almost €3.6 million (including fees and VAT, exclusive of rebates). There have been persistent issues with shortages of liraglutide since the introduction of the MAP, which has undoubtedly had a dampening effect on the cumulative expenditure. The MAP process does provide for an appeals mechanism for phase 1 or phase 2 applications that were not approved; where it can be demonstrated that patients could not access consistent supplies of liraglutide, it has been possible to extend the duration of reimbursement support to allow patients the opportunity to access continuous treatment.

> The successful rollout of this health technology management initiative in a high-demand therapeutic area in the primary care setting has allowed eligible patients to access a cost-effective treatment for weight management through their general practitioner.

It is possible to consider a scenario where there is no reimbursement application scheme in place, where we assume that all applicants are approved for reimbursement. In this scenario, expenditure over the same period could have been up to €15.4 million. This demonstrates the effectiveness of the MAP in allowing reimbursement support only in the approved cohort where cost-effectiveness has been demonstrated and the power of the MAP to contain expenditure.

Implications and lessons learned

The successful rollout of this health technology management initiative in a high-demand therapeutic area in the primary care setting has allowed eligible patients to access a cost-effective treatment for weight management through their general practitioner. This is in line with the long-term health system reform agenda, Sláintecare, which seeks to deliver care in the primary care and community settings where possible. The implementation of the RAS and MAP for liraglutide will likely have implications for reimbursement of other weight management medicines. The data collected will be available to better inform future reimbursement decisions. This initiative has demonstrated that it is possible to implement outcomes-based conditional reimbursement of a medicine on a national scale through our existing IT infrastructure.

The MAP has facilitated patient access while managing the expenditure, as illustrated in our counterfactual expenditure example above. As of December 2023, there were 28 medicines reimbursed subject to a RAS and MAP in Ireland, with more than 9400 applications for individual reimbursement approval reviewed by the HSE MMP in 2023. These medicines cover a broad range of therapeutic areas, are delivered across community and hospital settings, and include medicines like liraglutide with high utilization, but also a number of gene therapy products and other medicines for rare diseases.

Health technology management processes such as RAS and MAPs are now well-established in Ireland.[8,9] These processes are enabling patient access to innovative medicines while providing the decision maker with improved oversight and a greater degree of cost certainty, and they are likely to remain part of the reimbursement environment into the future.

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Declaration of interest

The authors have no conflicts of interest to declare.

Ethics

The local Research Ethics committee reviewed an ethics application for this study and determined that no ethical consideration/approval was necessary for the analysis of the anonymized data.

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What Must Change if Future Evidence Pricing and Access Challenges to Healthcare Technologies Are to Be Overcome Successfully?

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Major hurdles will need to be overcome if future healthcare technologies are to deliver value to all stakeholders (patients, payers, physicians, politicians, pharmaceutical companies, and diagnostic/digital device/software developers).

For successful delivery of affordable healthcare technologies, systemic changes (information technology systems, regulatory and health technology assessment systems, coding payment and funding systems, ethical and legal systems) will be required.

Strategic collaboration between manufacturers and data providers/brokers to access data, and integration of data from multiple sources (prescription data, electronic medical records, health resource utilization, claims' data, real-time/wearable data, social media data) will be major drivers of success.

Healthcare technologies are evolving rapidly but the systems used to support the assessment, appraisal, access, and funding of these technologies are evolving more slowly. This disparity in speed of evolution will create future evidence pricing and access challenges.

Using the ISPOR top 10 HEOR trends[1] as a catalyst, a 2-cycle delphi approach based on broad expert opinion (n=41) that included payer experts in immunology and oncology based in the European Union, the United States, and Asia Pacific and representatives from Ipsos' autoimmune and oncology centers of expertise was used to determine the likely key evidence pricing and access challenges over the period 2023-2030 and requirements for their solution.

> The research indicated that the challenges could rarely be overcome by companies working in isolation, or by limiting solutions to single data types or sources.

Scope covered the complete lifecycle pathway from drug discovery to loss of exclusivity (LoE); evolution of digital health technologies including predictive analytics and artificial intelligence (AI); patient selection informed by genomics; and value attribution in situations where value is delivered by multi-component disease management rather than by drugs or interventions in isolation.

Challenges

Cluster analysis was used to reduce the many challenges into 10 distinct "challenge clusters": affordability, evidence, assessment, pricing, differentiation, sequencing, personalization, value attribution, portfolio optimization, and pace of change.[5]

The challenges, which were applicable to most therapy areas, involved multiple stakeholders (physicians, payers, patients, pathologists, politicians, and pharmaceutical manufacturers). Capability requirements were identified that centerd around "willingness" (to pay, to change behaviors, to invest, to collaborate) and "ability" (to pay, to access, integrate, and accept data from multiple sources).

The research indicated that the challenges could rarely be overcome by companies working in isolation, or by limiting solutions to single data types or sources.

The largest challenge cluster related to "personalization" (digital and molecular diagnostics/biomarkers linked to therapeutics) and highlighted the key challenges that need to be addressed for digital health to be successful (**Figure 1**) and the barriers to effective use of molecular diagnostics and linked therapy

Figure 1. Key Challenges

Three key challenges need to be addressed for digital health technology to be successful[2]

First Challenge: Speed of evolution

Technology is evolving faster than the regulatory, behavioural, healthcare funding, and health technology assessment (HTA) systems that are required for successful implementation.

For digital health to deliver on the promise, developers will need to produce relevant robust evidence regarding the technology for assessors; systemic changes will be required in regulatory and HTA assessment systems, the roles of the physician and data in disease management, payment systems, and the pricing of healthcare will need to change.

Second Challenge: Evidence

There are various challenges to evidence development in this environment, including:

- Relevance, robustness, and rigor
- Difficulty and cost of evidence development
- Timeliness of evidence delivery
- Continued validity of evidence in a rapidly evolving environment
- Measurement and attribution of co-dependent value between developers of the different disease management components.

Third Challenge: Value

Value will be attributed and assessed in different ways:

- Value contribution of 3 different elements: monitoring, intervention, and prediction
- Value segmentation based on 3 outcome types: economic, clinical & humanistic
- Value perception based on 3 stakeholder groups: patient, payer & physician
- Value attribution, informing value-based reimbursement allocation, which will become increasingly important as stakeholders adopt more holistic disease management

use that will need to be removed for biomarker-driven healthcare to be successful (**Figure 2**).

Solutions

The research indicated that evidence pricing and access approaches will need to be re-engineered to address 4 factors: the evolution of science and technology, multicomponent disease management, multistakeholder value attribution, and multisource data integration.

Innovative approaches will increasingly be needed around evidence creation and the construction of pricing propositions. These will include multisource data modeling, conditional reimbursement/coverage with evidence development, financial and outcomes-based risk sharing, and innovative funding models drawn from other industries (eg, financial services) such as monthly, quarterly, or annual "per patient subscription" and "reverse discounting/endowment life insurance" to reflect the risk equation changing as certainty of outcome increases.

Solutions will require broad fundamental systemic, organizational, and stakeholder changes (**Figure 3**) to overcome the evidence availability, accessibility, assessment, and acceptability challenges. The implementation of these will take time and face considerable debate[6] and opposition as witnessed in the United States (around Medicare price negotiation within the Inflation Reduction Act) and in Europe (around Joint Clinical Assessment and the revision of the EU Pharmaceutical strategy).

> Innovative approaches will increasingly be needed around evidence creation and the construction of pricing propositions.

Strategic collaboration between manufacturers and data owners/providers will be required. Through such relationships, manufacturers will be able to more easily access comprehensive real-world data and real-time data, which they can utilize across their portfolios and in their interactions with payers. This does, however, raise potential additional ethical and legal challenges around data access, use, ownership, and patient rights. A recent example was Google and DeepMind facing claims for unauthorized and inappropriate National Health Service medical record use.[7] There may be a need for data brokers as intermediaries between pharma and healthcare systems to audit data quality and between healthcare providers and pharma to improve public acceptability of data sharing.

Financial engineering will be needed to address the challenges of cell and gene therapies and other technologies with high price density or single one-off initial costs. These present specific challenges around cost and affordability, funding flows, uncertainty around long-term benefits, and value definition.[4]

New payer-types will need to emerge. Examples might include creation of innovation "banks" utilizing money from the public purse, venture capital, and pharmaceutical and diagnostic companies that will invest in building the required infrastructure so that medical innovations can be introduced rapidly, while the financial arrangements are stabilized through consideration of attribution of value. Funding flows will need to change to relieve financial pressures. These are likely to include phased outcomes-based payments, expanded risk pools, annuities, mortgage/loans, re-insurance (where data would need to be shared with re-insurers in a similar way that pharma shares data with health technology assessment, to give them confidence that underwriting is possible), supplier credit, and direct payments. Any one or combination of these has the potential to incentivize payers, both traditional and new, to invest their limited financial resources.

Figure 2. Key Barriers

Eight barriers to molecular diagnostics and five to therapy use will need to be removed for biomarker driven healthcare to be successful[3]

Barriers to molecular diagnostic use may include:

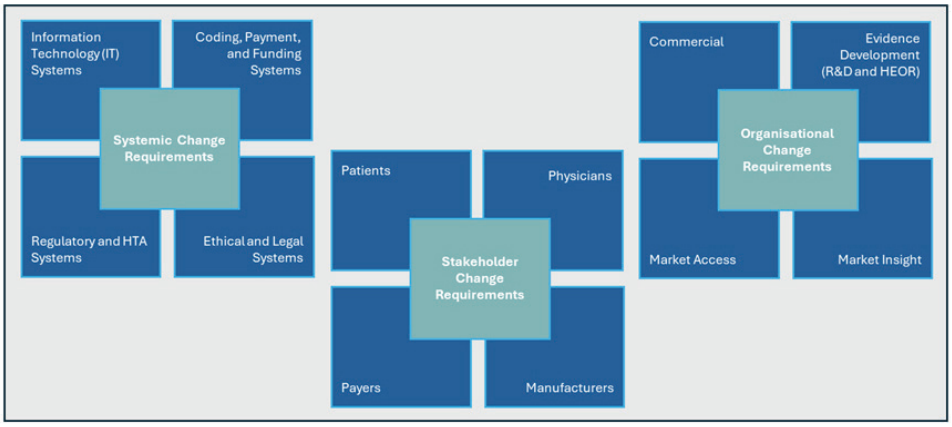
1. Funding
2. Access to and availability of testing
3. Testing methods and process: difficulty of obtaining sample, complexity, and turnaround time
4. Test performance: will the test be (or perceived to be) insufficiently accurate or ambiguous?
5. Population selected for testing: will the population tested be as broad as the drug's indication?
6. Physician's adoption of the test proposition
7. Patient demand for testing, and willingness to be tested
8. Conversion rate: will physicians prescribe other drugs despite a "positive" test result?

Barriers to therapy use may include:

1. Affordability, access and availability: of drugs, diagnostic testing, reimbursement, and data/evidence
2. Timing: delays in treating treatment guidelines, delays in Health Technology Assessments and their implementation, and time lag in adopting technology
3. Preference: influenced by context, personal experience, and outcomes of earlier treatment(s)
4. Policy & priorities
5. Power: Physician vs payer, HTA vs medical society, and treatment guideline perspectives differing from patients' perspectives.

Figure 3. Changes Required

Fundamental changes will be required to overcome the evidence availability, accessibility, and acceptability challenges



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Reimbursement of Digital Therapeutics: Deep Dive on the United Kingdom and Implications for Manufacturers

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Digital therapeutics offer significant promise for healthcare systems and patients, but they have struggled to achieve broad public reimbursement.

NICE launched the Early Value Assessment for MedTech in 2022 to help support digital therapeutics uptake. Under this pathway, digital therapeutics can receive conditional recommendations to support adoption contingent on future data collection.

Over 50 digital therapeutics have been assessed since its inception with over 30 recommendations, potentially providing a model value framework for other countries.

Lessons Learned

The United Kingdom's evolving approach to assess and reimburse digital therapeutics (DTx) offers important opportunities for manufacturers considering launching in Europe. The UK's Early Value Assessment (EVA) pathway, launched in 2022, demonstrates the government's intent to promote early adoption of promising DTx, particularly those addressing key national health issues. However, the pathway's reliance on conditional recommendations, contingent on the collection of real-world evidence (RWE), underscores the need for manufacturers to plan strategically.

One major lesson is the importance of preparing for evidence generation. While EVA facilitates quicker access to the National Health Service (NHS), it requires manufacturers to generate additional data to secure long-term reimbursement. Small- and medium-sized companies must be proactive in securing funding from available UK programs such as the NIHR i4i, NIHR, and OLS RWE Programme to support these efforts.

> Many healthcare systems are characterized by fragmented data environments, where information is siloed across different electronic health record systems, registries, and proprietary platforms.

Another key takeaway is the importance of differentiation to compete in a crowded space. Since the EVA groups DTx in Health Technology Evaluation (HTE) bundles, manufacturers must develop and communicate a compelling value proposition that highlights their product's unique benefits to stand out in a competitive landscape.

Lastly, the absence of a legal mandate for recommendations to be reimbursed country-wide means that securing NHS reimbursement requires strong relationships with regional NHS entities. Manufacturers should engage these stakeholders early in the process to facilitate adoption and ensure that their DTx achieves the desired access.

In summary, while the United Kingdom offers a promising pathway for DTx reimbursement, manufacturers must be diligent in planning for evidence generation, differentiation, and stakeholder engagement to succeed in this rapidly evolving market.

Digital Therapeutics Offer Huge Clinical Potential but Have Faced Significant Access Barriers to Date

The increasing prevalence of chronic diseases and aging populations are imposing significant challenges on public health systems, with financial pressures expected to intensify in the coming years. Digital therapeutics (DTx) present a promising solution to mitigate these economic challenges to healthcare systems as well as improve health outcomes for patients.

Despite the potential of DTx, many of these technologies are not yet profitable or are only marginally so. In Europe, a major issue is reimbursement by public health systems; demonstration of the clinical value of DTx using traditional health technology assessment (HTA) methods can be very challenging. HTAs rely on randomized controlled trials (RCTs) as the gold standard to demonstrate clinical benefit. However, conducting RCTs for DTx can be impractical due to factors such as difficulty in blinding participants, maintaining patient engagement, and identifying and measuring relevant health outcomes. Further, HTA methods were originally designed for static interventions such as medicine, but digital interventions can rapidly evolve, even

more so through artificial intelligence and machine learning, potentially making the intervention more cost-effective over time and necessitating re-evaluations. Accordingly, real-world evidence (RWE) is expected to play an important role in the evaluation of DTx, as it allows continuous assessment of their effectiveness in real-world settings.

There may be, however, notable challenges in the utilization of RWE to support the value of DTx. These include structural barriers that can hinder the collection and utilization of RWE. Many healthcare systems are characterized by fragmented data environments, where information is siloed across different electronic health record systems, registries, and proprietary platforms. These systems often lack interoperability, making it challenging to integrate and harmonize data for analysis. Moreover, variations in data collection practices across regions and institutions further complicate efforts to aggregate comprehensive datasets. Access to data is another obstacle, as policies governing data sharing and privacy may vary widely across jurisdictions. Even when RWE is collected, it often faces inherent limitations when compared to RCTs, such as the introduction of confounding biases due to nonrandomization and concerns about the reliability of data quality (eg, incomplete or omitted registry data). These issues can compromise the generalizability of findings, which may cause payers and HTA bodies to approach RWE with caution.[1]

Despite these challenges, the European DTx market size was $1.68 billion in 2023 and is expected to grow to $13.9 billion by 2033.[2] The DTx market in Europe is predominantly concentrated in a few nations that have adopted measures to evaluate and/or reimburse DTx (**Table**). This fragmented and complex market sees Germany as the most influential player, as they were the first in introducing a process for the reimbursement of DTx, the Digital Health Applications (DiGA) in 2020. Under this process, a single RCT is sufficient to obtain reimbursement, decisions are made within 3 months, and the average price for permanently listed DTx is €532.[3,4]

Table. Overview of the DTx frameworks, pathways, and funding across major European markets

Category	Belgium	Germany	France	Italy	Netherlands	Spain	Sweden	UK
National value assessment framework	✓	✓	✗	✗	✗	✗	✗	✓
National reimbursement pathway	✓	✓	✓	✗	✗	✗	✗	✗
Available funding mechanisms	✓	✓	✓	✗	✓	✓	✗	✓

After reimbursement, integrating DTx into the healthcare system may still face challenges, particularly among certain patient demographics. The uptake of DTx may be lower among groups such as those with low digital literacy, older adults, low-income populations, and rural residents. This may be especially concerning as people in these groups typically have greater healthcare needs. These factors influencing DTx uptake may thus hinder equitable adoption and achieving optimal healthcare outcomes.[5]

The UK Approach: Conditional Reimbursement Pending RWE collection

The UK established an Evidence Review Framework in 2019; however, only one product received a recommendation under this pathway, Sleepio in 2022, at a listed price of £45 per user.[3,6] During this period, 29 DTx had been permanently reimbursed in Germany.[7]

The UK subsequently adopted a distinct approach termed "Early Value Assessment (EVA) for MedTech" in 2022. EVA is designed to facilitate the early adoption of promising technologies addressing national health concerns within the NHS. Through EVA, technologies may receive a conditional recommendation for use, a recommendation for research only, or no recommendation. Those recommended for use will be subject to an evidence generation plan, with a subsequent full NICE assessment based on the new data generated. However, these recommendations have no legal mandate to be followed, although it is expected that they will help support local access.

As of August 7, 2024, 57 DTx had been evaluated by NICE under the EVA pathway in under 2 years. Fifty-two percent of the outcomes were conditional recommendations, 39% for research only, and 9% were not recommended.[8]

Considerations for Manufacturers

The EVA pathway has evaluated many DTx in the United Kingdom since its inception in 2022. Most EVA recommendations are conditional on future data collection. However, companies need to find a way to fund these evidence-generation plans. It is critically important, especially for small manufacturers, to leverage various funding programs to support data collection, such as the NIHR i4i, the NIHR, and OLS RWE Programme.

> The European digital therapeutics market size was $1.68 billion in 2023 and is expected to grow to $13.9 billion by 2033.

Under EVA, DTx are grouped in Health Technology Evaluation (HTE) bundles comprising multiple technologies with similar characteristics that target the same indication, and often with several DTx securing conditional recommendations. Therefore, competitive differentiation becomes a critical consideration, and development and dissemination of a compelling payer value story centered around the unique product characteristics becomes key.

Finally, the lack of a legal mandate for NICE recommendations under EVA means that manufacturers should establish and leverage strong relationships with local NHS entities early to help secure local reimbursement.

In conclusion, DTx offer the potential for substantial patient and healthcare system benefits, but manufacturers need to plan and prepare appropriately to navigate the rapidly evolving technology appraisal, coverage, and funding dynamics between different markets to ensure these DTx innovations can support healthcare ecosystems in providing timely and appropriate care for patients who so urgently need them.

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The Health ROI Assessor: Evaluating Novel CVD Prevention Approaches in Estonia

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Investing in prevention has been considered historically as a cost factor, ignoring that effective health investments induce wealth creation in terms of gross value added and employment.

The proposed Health ROI Assessor Framework introduces a new concept of value assessment, uniting health-related human capital effects and investment effects both within and beyond the health sector.

The cardiovascular disease prevention program in Estonia generates substantial health and economic gains, particularly when intervening in younger age groups.

Introduction

Substantial evidence supports the link between improved health, economic growth, and well-being across countries.[1-6] However, there's been limited progress in advocating for increased national budget allocation to the health sector because, despite the evidence, health investments have been viewed historically as an economic burden rather than a driver of growth. The absence of empirical evidence on the pathway that links a particular investment in health and the resulting economic growth and a lack of frameworks and metrics estimating return on investment (ROI) contribute to this challenge.

> There's a lack of consistent efforts to predict the total ROI of health expenditures holistically, leaving current research results on rhetorical explanations with limited use in informing policy decisions.

Concern arises when health investments, such as those related to population prevention programs, demand a significant portion of the health sector budget. In such cases, policymakers grapple with limited budgets and imperfect information. The current health technology assessment (HTA) tools, primarily designed for curative care, inadequately capture the value for money in preventive measures like screening or vaccination, adding to the difficulty.[7-9]

In light of recent economic challenges stemming from the COVID-19 pandemic, there's a renewed interest in understanding how health investments contribute to economic growth and stability. Current research highlights 3 main mechanisms: first, health investments drive innovation for better disease management, reducing healthcare costs.[10-14] Second, a healthier population is more productive.[3,4,12,14] Third, health investments impact the broader economy by influencing supply and demand across sectors.[15,16] Despite these insights, there's a lack of consistent efforts to predict the total ROI of health expenditures holistically, leaving current research results on rhetorical explanations with limited use in informing policy decisions.

We propose an innovative approach entailing a societal and macroeconomic perspective to assess value creation of health investments promoting countries' economic growth: the Health ROI Assessor framework that we operationalize in a measurement tool. This study applies the Health ROI Assessor for the first time to evaluate the ROI of a real-world prevention program in Estonia, proposed by the Prevent Cardiovascular Disease Coalition (P-CVD). We assessed the value creation of health investments reducing cardiovascular disease (CVD) burden and promoting economic growth within the country. Estonia has one of the highest CVD event rates in Europe and a comparatively high prevalence of CVD risk factors, which lead to high direct and indirect costs.[17,18] In 2019, CVD accounted for over 50% of adult mortality and over 38,000 hospitalization cases.* The P-CVD program provides a tailored solution to reduce low-density lipoprotein cholesterol (LDL-C), where CVD event risk profiles consider the cumulative exposure to LDL-C of the considered population (males/females, ages 30-79 years, without CVD history).

Methodology

The framework includes 2 pillars (**Figure 1**): **The Health Economy (HE) Footprint** estimates direct effects within the HE (gross value added [GVA] and corresponding job creation) and spillovers to other economic sectors (indirect/induced effects). The analysis builds on multiregion input–output data, tracing the value chain of health throughout economies[19-21] to estimate the magnitude of the health sector. **The Human Capital Effect** evaluates gains in productivity related to better health outcomes, incorporating paid and unpaid work participation. Labor-market interactions

connect both pillars via labor-force supply/demand adjustments.

Model inputs include age-, gender- and risk-specific previously estimated avoided CV events based on the results of a cost-effectiveness model implemented in a separate project.[22] Time series analysis allows us to build a dynamic framework of health investments. The implementation is based on health and economic data from official statistics and considers a discount rate of 3.5%.

We evaluated 2 scenarios: the base case scenario assesses short-term impacts over 15 years, and an alternative scenario examining long-term effects on the population initiating the P-CVD program in the 30-39 years of age cohort. The latter scenario focuses on the human capital pillar, extending to an estimated productive life expectancy of 85 years. Furthermore, it assumes that economic variables will remain constant beyond the initial 15 years, mirroring values predicted in the latest forecasted year.

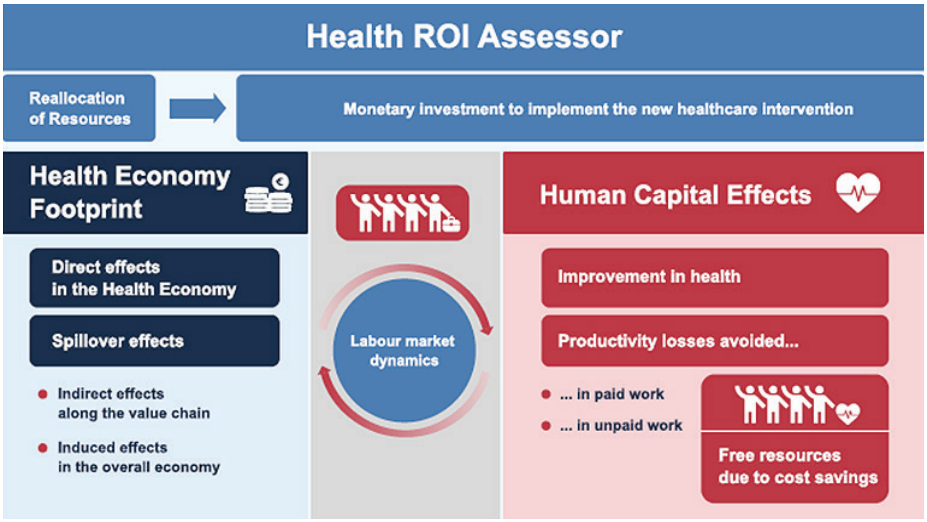
Results

Base case scenario – Short-term effects

Results illustrate considerable ROIs from the P-CVD program. For the HE Footprint (**Figure 2**), we assume a value of €20 million additional funding into the health system to implement the intervention, distributed equally over 5 years. This capital injection induces €46 million GVA and 1177 additional jobs in direct, indirect, and induced effects over 10 years.

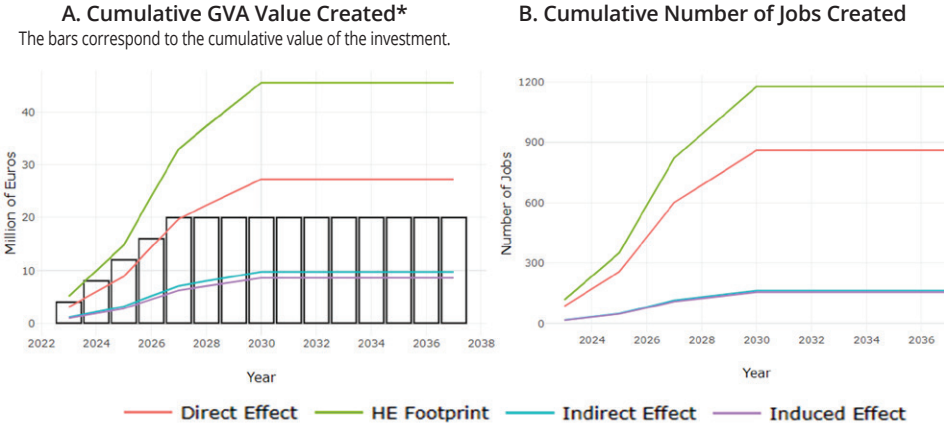
Regarding the Human Capital Effects, for a population of 50,527 individuals participating in the P-CVD program, P-CVD prevents 315 deaths and 928 nonfatal events over 15 years. This translates to an increase in paid (€47.97 million) and unpaid work (€61.20 million) productivity of €109.18 million. Given higher CVD incidence in older age cohorts, the short-term CV events avoided are less for younger cohorts. Therefore, the highest paid work productivity gains occur for the 50-69 years of age cohorts (**Figure 3A**). Additionally, Figure 3B displays that the differences in unpaid work productivity gains are more pronounced between the older and younger age cohorts than those observed in Figure 3A, reflecting varying time utilization patterns.

Figure 1. Health ROI Assessor framework



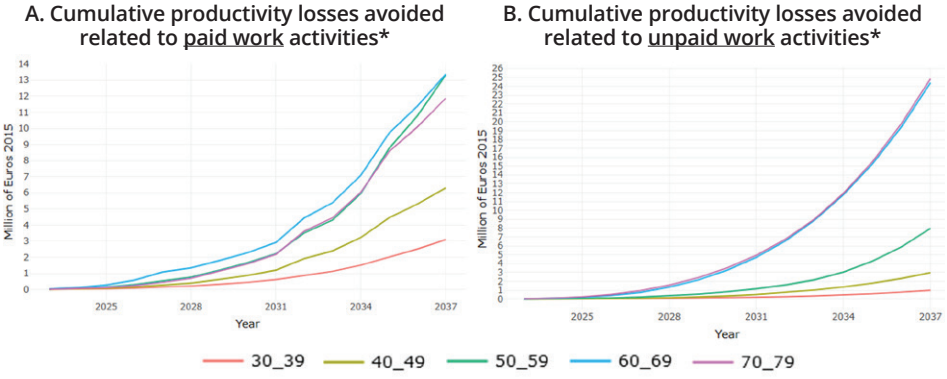
Source. Authors Elaboration

Figure 2. Short-term: Cumulative value created in HE Footprint pillar (first 15 years)



**Discount rate 3.5%; Abbreviations: GVA, gross value added; HE, health economy*
Source. Authors Elaboration

Figure 3. Short-term: Cumulative value created** by age cohort in the Human Capital Effects pillar (first 15 years)

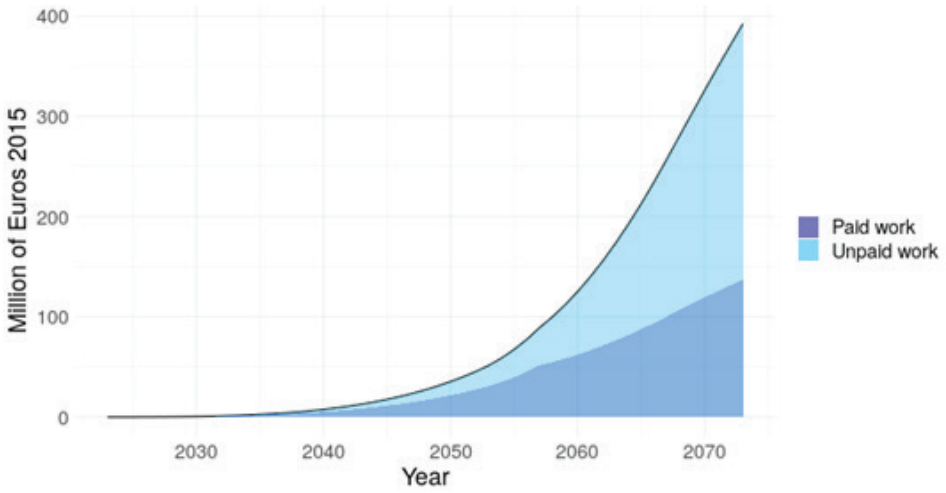


** Lines represent the age cohorts participating in the P-CVD program. **Discount rate 3.5%.*
Abbreviations: P-CVD, Prevent Cardiovascular Disease Coalition
Source. Authors Elaboration

Alternative scenario – Long-term effects resulting from the Human Capital Effects pillar

Assuming a maximum productive lifespan of 85 years, enrolling a cohort of 12,077 individuals aged 30–39 in the P-CVD program results in a gain of 13.35 million productive hours. It translates into €392.35 million of value creation (GVA), where the majority corresponds to productivity gains in unpaid work (**Figure 4**).

Figure 4. Long-term: Cumulative value created* in the 30 to 39 years of age cohort along their productive lifespan



**Discount rate 3.5%*
Source. Authors Elaboration

Discussion

The Health ROI Assessor framework is the first to offer a comprehensive perspective on the overall value of healthcare intervention programs, complementing conventional economic evaluations. This is particularly crucial for impactful interventions with substantial investments. For example, prevention programs yield long-term benefits by contributing to human capital formation and creating a cascade of effects that start in the health sector and affect the entire economy.

Results suggest a substantial economic impact of implementing the P-CVD program in Estonia, even in the short-term (first 15 years). The HE Footprint pillar suggests a ROI in terms of GVA contribution equal to 2.3 times the amount invested in the P-CVD program when capturing effects along the entire value chain. The Human Capital Effect indicates an increase in productivity of €109 million, which corresponds to around 0.4% of Estonia's GDP in 2023 (2015 prices).

While short-term productivity gains in younger populations are modest, the long-term cumulative impact is significant. The youngest cohort (30-39 years old) experiences a reduction in CVD events that results in €392 million productivity gains, equivalent to 1.6% of Estonia's GDP in 2023 (2015 prices). This underscores the economic value of investing in early prevention, especially targeting younger age groups, for sustained long-term growth.

The proposed framework offers key advantages. It links macroeconomic investment perspectives with microeconomic social impact evaluations, providing a holistic view of improved population health. Unlike traditional methods, we monetize using sector-specific GVA per working hour instead of wages, enhancing the accuracy in reflecting the value of labor productivity. By using national accountability data and publicly available economic variables, our results can be integrated into a country's accounting system. Furthermore, the methodology's adaptability makes it applicable to diverse countries and healthcare interventions, facilitating comparisons and evaluations. The Human Capital Formation addresses the impact of a healthier population, emphasizing productivity as a key factor. This is crucial to counter the alarming decline in the percentage of individuals in the working-age population, which represents a substantial threat to future financial and fiscal sustainability.[23-27] Additionally, the capacity for unpaid work, an often-overlooked aspect crucial for economic well-being and societal health, is considered. Unpaid work is estimated to exceed 40% of GDP in some countries,[28] disproportionately affecting women's participation in the paid economy[29] and constituting a significant component of older adults' contributions to economic growth.[30] Finally, by considering input–output methods, our framework allows policymakers to compare the healthcare and other economic sectors in terms of the economic gains of injecting capital into the system.

The initial version of the Health ROI Assessor has limitations typical of a first modeling attempt, which will be captured in an enhanced version in development. First, predictions rely on available data and applied assumptions. Second, while it addresses relevant macroeconomic and societal effects, some aspects, such as the productivity of informal caregivers, are not included. Third, direct cost savings in the healthcare system from reduced treatment demand are not factored in. However, this dimension has been addressed in the work of Clayson et al.[22] For the intervention program under consideration, they report a total direct cost reduction of €323,757 per 1000 individuals, which is roughly 51%. In a future version of the ROI Assessor framework, we intend to incorporate these gained resources in an additional stream of value creation.

Conclusion

Results suggest substantial economic effects of implementing the P-CVD program in Estonia. The highest accumulative economic value creation is achieved when the intervention affects populations at younger ages.

The Health ROI Assessor framework offers an innovative approach to simultaneously evaluate the human capital and economic effects of healthcare interventions. Aligned with the G20 partnership for health and development's call for a more structured approach to health investment

assessment, our framework addresses this need. Amid ongoing global challenges, our tool stands out as a valuable addition to economic evaluation tools, aiding policymakers in optimizing resource allocation for enhanced population well-being. Shifting from a cost-centric viewpoint, the framework contributes to an understanding of public health and related interventions as a motor of economic and societal growth.

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Data Availability

Data provided by the Estonian National Institute for Health Development (NIHD /TAI) upon request.

Detailed methodology for the Health ROI Assessor is available upon request.

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Shaping a More Sustainable and Resilient European Healthcare System

Interview With Stephan Eder, President, Medicines for Europe

Q&A

European pharma is at a turning point, with rising cost pressures, regulatory shifts, and supply chain vulnerabilities reshaping the industry. As the new president of Medicines for Europe, STADA's Stephan Eder is now at the forefront of driving the change that Europe needs. In this new role as head of the leading EU-level lobby group for the generics, biosimilars, and value-added medicines industries, Eder argues that long-term sustainability requires moving beyond price-driven policies to recognize the sector's broader role in innovation, resilience, and patient access.

"Medicines for Europe is committed to balancing patient access with sustainability, ensuring that regulatory frameworks support long-term resilience while addressing environmental considerations."

– Stephan Eder

PharmaBoardroom: What led you to assume the presidency of Medicines for Europe, and what are your key priorities in this role?
Stephan Eder: Stepping into this role is both a responsibility and an opportunity to drive policies that safeguard patient access to essential medicines while reinforcing Europe's pharmaceutical industry. Representing STADA, I see this position as an extension of our broader obligation to collaborate with industry leaders in shaping a more sustainable and resilient healthcare system. The challenges we face—ranging from supply security to evolving regulatory frameworks—require a unified approach to ensure that patients continue to receive high-quality, affordable medicines without disruption.

Our priorities focus on 3 key areas. First, we must strengthen long-term, sustainable access to medicines, including generics, biosimilars, and value-added treatments. Generic medicines already account for 7 out of 10 prescriptions in Europe, and biosimilars have significantly improved access to biologic therapies, allowing earlier intervention for serious diseases and enhancing patient outcomes. Ensuring that policy frameworks fully recognize the critical role these medicines play in healthcare systems is essential for their continued availability.

Second, we need to reinforce Europe's role as a global pharmaceutical manufacturing hub. The off-patent medicines sector comprises around 400 manufacturers, directly employing 200,000 people and supporting extensive supply chains. A strong, coordinated EU industrial policy—one that includes both member states and candidate countries—is vital to securing Europe's pharmaceutical sovereignty, stabilizing supply chains, and bolstering production capabilities in key areas such as active pharmaceutical ingredients, biosimilars, and generics.

Finally, advancing a strong environmental, social, and governance agenda remains a priority. Medicines for Europe is committed to balancing patient access with sustainability, ensuring that regulatory frameworks support long-term resilience while addressing environmental considerations. Striking this balance will be key to maintaining a competitive, responsible, and future-proof pharmaceutical industry. By focusing on these priorities, we aim to drive meaningful progress that benefits patients while strengthening Europe's position as a leader in healthcare innovation.

PB: How should Europe's pharmaceutical legislation evolve to secure long-term sustainable access to medicines?
SE: A well-calibrated regulatory framework is essential to ensuring that medicines remain accessible, affordable, and sustainable across Europe. As the revision of EU pharmaceutical legislation progresses, key improvements are needed to eliminate inefficiencies, harmonize market entry conditions, and support a more flexible and responsive supply chain.

One of the most pressing issues is the harmonization of the Bolar Exemption Clause, which allows manufacturers to complete regulatory approvals, pricing negotiations, and reimbursement procedures before a drug's exclusivity period expires. Currently, discrepancies in how this exemption is applied across EU member states create inconsistencies, leading to delays in market entry. A uniform approach is essential to ensuring that once a patent or regulatory exclusivity ends, generic and biosimilar alternatives can enter the market without delay, immediately expanding patient access.

> For Europe to remain competitive while upholding both sustainability and access to medicines, environmental policies must be holistic and aligned with broader healthcare priorities.

Another important step would be the adoption of an Electronic Patient Information Leaflet (ePIL). Under current regulations, medicines must be distributed with printed leaflets in the official language of each country, complicating cross-border movement and creating inefficiencies in supply chains. A digital leaflet, accessible via a QR code, would allow patients to access information in their preferred language, select larger fonts for readability, and obtain simplified versions for better comprehension. Beyond improving patient experience, an ePIL would enable medicines to be redistributed across markets without the need for significant physical packaging modifications, reducing supply disruptions and strengthening access. Despite strong public and industry support, EU regulations still mandate printed leaflets, an outdated requirement that must be reconsidered.

Finally, market exclusivity periods must strike the right balance between rewarding innovation and ensuring timely competition. The European Commission's proposal to cap regulatory data protection at 11 years provides a reasonable framework, maintaining incentives for pharmaceutical innovation while preventing prolonged monopolies that delay access to affordable alternatives. Extending exclusivity periods beyond this threshold would undermine competition and restrict cost-effective treatments for patients.

By streamlining regulatory pathways, modernizing patient information practices, and maintaining a balanced exclusivity framework, Europe can create a pharmaceutical ecosystem that fosters both innovation and accessibility, ensuring that medicines remain available, affordable, and sustainable for the long term.

PB: How can Europe ensure sustainable pricing for off-patent medicines while preserving competition and access?
SE: The off-patent pharmaceutical industry plays an essential role in expanding patient access and reducing healthcare costs by introducing competition once exclusivity periods expire. However, the relentless downward pressure on prices, compounded by rigid procurement policies, is a challenge for the economic viability of especially older, long-established generic medicines, thus posing a risk for patient access to such medicines. In many European markets, manufacturers face strict pricing regulations that prevent adjustments, even as production costs rise. This has led to companies withdrawing medicines or exiting certain markets entirely, reducing supplier diversity and contributing to shortages.

To safeguard access while maintaining economic viability, Europe must transition from a price-driven procurement model to a value-based approach that considers long-term sustainability. While cost savings have been substantial—biosimilars alone generated €10 billion in savings in 2023—an excessive focus on price is undermining supply stability and consequently access. A more balanced system should incorporate Most Economically Advantageous Tender criteria, which evaluate not only price but also supply chain resilience, environmental impact, and supplier diversity. Moving away from a winner-takes-all model, which concentrates supply in the hands of a single company, would strengthen competition and prevent supply disruptions.

This approach is a core pillar of the Critical Medicines Alliance, which is shaping the forthcoming Critical Medicines Act to secure the long-term availability of essential medicines. Nordic countries have already adopted more advanced procurement frameworks that integrate sustainability criteria, and broader implementation across Europe would help preserve a plurality of suppliers, mitigate shortages, and ensure a resilient pharmaceutical market—while still delivering cost efficiencies to healthcare systems.

PB: What progress has been made on the Critical Medicines Act, and why is it essential for Europe's pharmaceutical supply chain?
SE: The Critical Medicines Act is progressing swiftly, with the European Commission aiming to present a legislative proposal by March 11, within the first 100 days of the new mandate. Led by the Critical Medicines Alliance, a coalition of industry representatives, NGOs, and EU member states, this initiative seeks to strengthen Europe's pharmaceutical sovereignty. Adrian van den Hoven, Director General of Medicines for Europe, is co-chairing one of the working groups shaping its development.

The proposal is centered around 3 strategic priorities. The first is boosting pharmaceutical manufacturing within Europe (ie, the European Union and candidate countries). While the region remains a key production hub, the industry's expansion has to a significant degree shifted to Asia over the past 15 years. And I would like to emphasize that it is not about getting everything back to Europe, that would be misguided and practically not possible. As an industry, we advocate partnerships across the globe in the production of medicines. Thus, it is about striking a balance and having robust and diverse supply chains both in Europe and outside of Europe. Therefore, the act aims to incentivize investment in European-based manufacturing, supporting both the expansion of existing sites and the development of new facilities. However, EU state aid regulations limit direct financial support, making private-sector funding essential. Given Europe's higher regulatory and environmental costs, investment in advanced technologies, digitalization, and sustainable production processes will be critical to ensuring competitiveness.

The second priority is reforming procurement practices to improve supply chain resilience. Currently, many generic medicine tenders rely on a lowest-price, single-winner system, which, while cost-effective, increases the risk of shortages if the sole supplier encounters production challenges. The act proposes a transition to multi-supplier contracts, ensuring a more diversified and stable supply. Additionally, supply chain resilience criteria would be introduced, giving preference to manufacturers with robust production capabilities. While procurement remains a national responsibility, establishing harmonized guidelines across the EU will be crucial for long-term sustainability.

The third and most complex issue is national stockpiling policies. Countries such as France and Germany have imposed extensive stockpiling requirements, with Germany alone mandating a 6-month reserve, equivalent to the annual consumption of 10 smaller nations in the vicinity of Germany. If more large economies implement similar policies, it could destabilize supply chains for smaller countries. Medicines for Europe, alongside several smaller EU states, is advocating for a more balanced approach and making it, also from a regulatory perspective, easier for companies to redistribute medicines in response to shortages rather than having reserves locked within individual national borders.

By reinforcing manufacturing, optimizing procurement, and ensuring fair shortage prevention practices, the Critical Medicines Act aims to secure a more resilient, competitive, and sustainable pharmaceutical ecosystem, ensuring reliable access to essential medicines across Europe.

PB: How can Europe turn stringent environmental regulations into a competitive advantage while safeguarding medicine access?
SE: Environmental, social, and governance principles are at the core of the pharmaceutical industry, with expanding access to medicines itself being a key objective. The industry is committed to sustainable manufacturing while ensuring the continued affordability of essential treatments. However, balancing ambitious environmental policies with pharmaceutical competitiveness and long-term medicine access remains a complex challenge.

Some European countries, particularly in the Nordic region, have already integrated environmental sustainability criteria into pharmaceutical procurement, emphasizing supply chain resilience, security of supply, and sustainable production alongside pricing considerations. This approach aligns with the broader objectives of the Critical Medicines Act, which seeks to establish more sustainable procurement frameworks across the EU.

> Our ambition is to elevate the discussion beyond cost considerations and ensure that the pharmaceutical sector is recognized for the value it brings in expanding patient access, driving innovation, and strengthening healthcare systems.

Yet, while strict environmental regulations can drive industry innovation, they must be carefully structured to avoid unintended consequences. The Urban Wastewater Treatment Directive is a prime example. This regulation proposes shifting the costs of quaternary water treatment onto the pharmaceutical and cosmetics industries, even though the issue at hand is medicine residues from patient consumption, not industrial emissions. What I would like to clarify: It is not about factory waste, which the pharmaceutical sector already manages through strict environmental standards. This policy disproportionately impacts high-volume, essential medicines such as cardiovascular and diabetes treatments, making them less economically viable. This could lead to market withdrawals, increasing shortages rather than addressing them. In short, it is a tax on medicine consumption for patients who are prescribed these medicines by their doctors to treat an illness, and some medicines might simply disappear from the market.

For Europe to remain competitive while upholding both sustainability and access to medicines, environmental policies must be holistic and aligned with broader healthcare priorities. A regulatory framework that balances innovation, supply resilience, and environmental responsibility will ensure that pharmaceutical production remains sustainable without compromising patient care.

PB: How are geopolitical shifts reshaping Europe's pharmaceutical industry and global partnerships?
SE: The pharmaceutical industry operates within a deeply interconnected global framework, relying on diverse supply chains, international manufacturing, and strategic partnerships. Recent geopolitical challenges—including COVID-19, the war in Ukraine, and growing protectionist policies—have intensified discussions around supply chain security. However, rather than advocating for complete pharmaceutical self-sufficiency, industry leaders emphasize the need for a balanced approach that reinforces European manufacturing while maintaining strong global cooperation.

The Critical Medicines Act and Critical Medicines Alliance reflect this shift, prioritizing investment in domestic production, particularly for generics and biosimilars, while recognizing that Europe cannot and should not operate in isolation. Expanding manufacturing facilities within the EU and in candidate countries like Serbia is a strategic step, yet collaboration with key partners in India, China, Vietnam, and the United States remains essential for securing active pharmaceutical ingredients, raw materials, and finished products.

At the same time, Medicines for Europe remains committed to global solidarity, resisting protectionist pressures that threaten medicine access. During COVID-19, when certain countries sought to restrict exports, the industry opposed such measures, reinforcing the principle that pharmaceutical supply chains must prioritize patient needs over isolationist policies. This international commitment is also reflected in industry leadership. Lucas Sigman, CEO of Insud Pharma, now chairs the International Generic and Biosimilar Medicines Association while continuing to serve on Medicines for Europe's executive board, demonstrating that European pharmaceutical resilience and global collaboration are not mutually exclusive but fundamentally interconnected. In an evolving geopolitical landscape, the focus remains clear: building a more diversified, resilient supply chain that integrates European production strength with global partnerships, ensuring long-term stability and uninterrupted access to essential medicines.

PB: What legacy do you hope to leave as President of Medicines for Europe by 2027?

SE: A meaningful legacy is not built on individual achievements but on what we, as an industry, accomplish together. Our ambition is to elevate the discussion beyond cost considerations and ensure that the pharmaceutical sector is recognized for the value it brings in expanding patient access, driving innovation, and strengthening healthcare systems. While affordability remains fundamental, it must not overshadow the critical role our industry plays in long-term healthcare sustainability and treatment advancements. An important role in this context is with value-added medicines, which enhance existing treatments through new indications, improved formulations, or combination therapies. In areas like cardiovascular care, combination treatments improve adherence and significantly enhance patient outcomes, demonstrating that pharmaceutical innovation is not only about new drug development but also about maximizing the impact of established therapies.

By 2027, I want our industry to be seen not just as a cost-saving sector but as an essential partner in healthcare—one that policy makers and patients can rely on to ensure sustainable access, advance treatment effectiveness, and drive long-term medical progress across Europe.

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