SEPTEMBER/OCTOBER 2025 VOL. 11, NO. 5

VALUE & OUTCOMES SPOTLIGHT

An HEOR news magazine

UPSKILLING:

The New HEOR *Imperative*

ISPOR

Improving healthcare decisions

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SEPTEMBER/OCTOBER 2025
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An HEOR News Magazine

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.

ISPOR
Improving healthcare decisions

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***VALUE & OUTCOMES SPOTLIGHT* EDITORIAL OFFICE:**

Value & Outcomes Spotlight
Online: ISSN 2375-8678
Published bimonthly by:
ISPOR
505 Lawrence Square Blvd, S
Lawrenceville, NJ 08648 USA

Direct photocopy permission and reprint requests to Director, Publications.

Cover photo courtesy of AdobeStock/Sina Blaze

Empowering the HEOR Workforce for a Dynamic Future

The field of health economics and outcomes research (HEOR) is undergoing an unprecedented transformation. From regulatory shifts driven by joint clinical drug assessments and emerging digital health ecosystems to the innovative use of artificial intelligence (AI), the HEOR landscape today is as complex as it is exciting. In this rapidly evolving field, empowering the HEOR workforce is not just beneficial but essential for the sustained delivery of impactful research that can resonate across all levels of healthcare.

Professional Development: Fostering Future Talent

One undeniable truth is that continuous learning lies at the heart of every dynamic profession. For HEOR professionals, keeping abreast of regulatory changes, advanced methodologies, and cutting-edge technologies has become critical. Companies have recognized this need by establishing robust training programs and professional development initiatives. These initiatives combine technical upskilling with hands-on practical training, from learning the latest AI advancements to mastering the interpretation of real-world evidence. Academic partnerships with universities and research institutions further enrich this ecosystem, ensuring that even new entrants have access to mentorship programs and internship opportunities that lay a strong foundation.

> In this rapidly evolving field, empowering the HEOR workforce is not just beneficial but essential for the sustained delivery of impactful research that can resonate across all levels of healthcare.

Continuous learning also involves soft skills development. With HEOR professionals increasingly embedded in cross-functional teams that include Market Access, Medical Affairs, and other strategic units, effective communication and stakeholder engagement have emerged as prized competencies. As noted by industry leaders, effective translation of complex economic modeling into compelling narratives can dramatically influence payer decision making and medical strategy. By integrating soft skills training alongside technical development, HEOR organizations are cultivating versatile professionals equipped to navigate the dual demands of rigorous analysis and relatable, actionable communication.

Cross-Functional Collaboration: Breaking Down Silos

Traditional models of isolated HEOR groups are giving way to cross-functional collaborations, essential to the field's evolution in today's integrated healthcare environment. As HEOR functions merge with other teams, there is a unique opportunity to create a holistic approach to evidence generation, one that considers the broad spectrum of stakeholders from regulators to clinicians and patients. Cross-functional collaboration not only fosters more relevant and timely evidence but also enhances innovation.

Collaboration is not limited to internal organizational strategies. Industry experts advocate for partnerships that extend beyond the corporate sphere, bringing together academia, regulatory bodies, and even international healthcare networks. These collaborative endeavors can streamline the drug evaluation process and ensure that evidence generation projects are aligned with both patient care and payer expectations. In essence, by breaking down traditional silos, HEOR teams can accelerate the translation of research into actionable insights.

Embracing Advanced Technologies: AI and Digital Innovations

The transformative power of AI cannot be overstated. As HEOR teams integrate AI into their daily routines—from literature reviews to complex economic decision-making models—the result is a more efficient and comprehensive analysis process that saves time while enhancing accuracy. AI's ability to sift through thousands of data points or

generate detailed economic landscape reviews not only streamlines research but also allows HEOR professionals to focus on higher value endeavors.

In addition to AI, cloud-based platforms and digital tools are reshaping how HEOR data are stored, analyzed, and disseminated. With these technologies, professionals can collaborate in real time across geographies, ensuring that every team member has access to the latest data and insights. This digital transformation supports a more agile working model, one that aligns with the fast-moving nature of healthcare markets and new drug development pipelines. However, the integration of advanced technology must always be balanced with diligent oversight, ensuring that human expertise guides AI outputs to maintain scientific validity.

Work-Life Balance: The Human Element of HEOR

Empowering HEOR professionals is not solely a matter of academic and technological advancement; it also requires nurturing the well-being of the workforce. The pandemic has taught us that flexibility in working arrangements is vital. While remote work has provided many HEOR professionals with greater autonomy and work-life balance, it has also underscored the importance of in-person interactions for mentoring, collaboration, and professional growth.

With a blend of robust professional development, cross-functional collaboration, advanced technology integration, and thoughtful work-life initiatives, the HEOR field is well-positioned to not only meet–but exceed–the demands of modern healthcare.

Organizations are increasingly investing in comprehensive well-being programs that cater to their global teams. From country-specific wellness initiatives and virtual social events, these programs aim to create an environment where HEOR professionals can thrive. Such initiatives ensure that while the workforce is empowered with state-of-the-art tools and skills, they are also supported in maintaining a healthy, balanced lifestyle—a critical factor in sustaining long-term productivity and job satisfaction.

A Vision for the Future

As we look to the future, the evolving role of HEOR professionals in the workforce is clear. With a blend of robust professional development, cross-functional collaboration, advanced technology integration, and thoughtful work-life initiatives, the HEOR field is well-positioned to not only meet—but exceed—the demands of modern healthcare. Empowered by training and technology, and supported by a balanced work environment, HEOR professionals are set to become the architects of healthcare value, shaping public policy, influencing clinical practice, and ultimately, improving patient outcomes.

By embracing these imperatives, HEOR teams are transforming challenges into opportunities and ensuring that the field continues to deliver critical insights and value to make an everlasting impact in a rapidly evolving healthcare landscape. By reinventing and investing in our HEOR workforce, we can empower them to lead the way to a healthier future!

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

Workforce Planning and Development to Advance HEOR

Rob Abbott, CEO & Executive Director, ISPOR

An often overlooked—but fundamentally important—aspect of advancing health economics and outcomes research (HEOR) excellence is creating the conditions in which the HEOR workforce can thrive. This can take many forms. Professional development programs can enhance specific workforce skills. Cross-functional collaborations can foster innovation. The use of advanced technology can improve efficiency. Each of these, and more, is explored in this issue of *Value & Outcomes Spotlight*.

Professional development programs are at their best when they provide opportunities to learn new methods, update existing knowledge, and develop critical competencies such as data analysis, cost-effectiveness modeling, and stakeholder engagement and communication. The ways in which this is done are many and include targeted training, workshops, conferences, mentorship, and the creation of structured career pathways that foster continuous learning. Perhaps the most important thing that can be said about professional development is that it is not a "one and done" kind of thing; HEOR practitioners must continually adapt to the rapidly evolving demands of our field.

Specific ways in which professional development can enhance—and advance—HEOR include:

Targeted Skill Acquisition
Perhaps the most common and intuitively obvious expression of professional development are programs that advance skill acquisition in specific HEOR areas such as advanced statistical modeling, real-world evidence (RWE), and cost-effectiveness analysis.

Knowledge Update and Refreshment
The HEOR landscape is evolving rapidly, and professional development programs hold considerable promise as fora within which professionals at all stages of their career can learn new approaches, stay current with emerging trends, and refresh their understanding of key subjects.

Hands-On Experience
Work-based training provides hands-on experience within an HEOR setting, strengthening practical skills, and contributing to institutional capacity.

Networking and Collaboration
Conferences, seminars, and workshops provide opportunities for professionals to connect with peers, share insights, and build networks that foster collaboration and life learning. This is something I have observed first-hand through ISPOR conferences and related events.

Soft Skills Development
Alongside the overtly technical skills highlighted above, professional development can enhance critical "soft skills" such as communication, collaboration, and leadership, all of which are vital for effective HEOR research, implementation, and dissemination, particularly to nonexpert audiences.

Cross-functional collaboration can foster HEOR innovation by breaking down silos, combining diverse perspectives from clinical, data science, and business, and improving communication and integration across teams. This integration leads to better problem-solving, increased creativity, more efficient resource allocation, and faster development of innovative strategies to enhance patient care.

> Perhaps the most important thing that can be said about professional development is that it is not a "one and done" kind of thing; HEOR practitioners must continually adapt to the rapidly evolving demands of our field.

Equipping the HEOR workforce with advanced technology, particularly artificial intelligence and digital tools, can improve efficiency by automating repetitive tasks, enabling faster and more accurate data analysis, facilitating enhanced collaboration and knowledge sharing, and providing real-time access to critical information. This enables and empowers HEOR professionals to focus on higher-level cognitive work, leading to more informed decision making, improved productivity, and a greater capacity to deliver timely and specialized reports and insights.

As the CEO of ISPOR, I spend a good deal of time thinking about the path that HEOR is traveling through time and how ISPOR can create the conditions in which HEOR professionals can truly thrive. Workforce planning and development are important considerations in this regard. This issue of *Value & Outcomes Spotlight* comes at a critical time for our membership, which really is the global HEOR community. After all, many HEOR departments in global biopharma companies have been restructured over the past 18 months, and some of the changes to healthcare introduced or proposed by the White House create new challenges for HEOR professionals. Against this backdrop, I want ISPOR members and stakeholders to know that I care about these issues, and that ISPOR will keep taking action to ensure we are providing the professional development, cross-functional collaborations, and access to advanced technology to keep HEOR professionals relevant and impactful.

HEOR NEWS

1 Institute for Clinical and Economic Review Releases Draft Evidence Report on Treatments for Obesity (ICER)

The report concludes semaglutide and tirzepatide are cost-effective for obesity management in various healthcare settings—but with more than 92 million eligible patients in the United States and about 30,700,000 patients over 5 years initiating treatment with injectable or oral semaglutide or tirzepatide, this high demand will cause budget strain. ICER calculates the 5-year annualized potential budget impact threshold that should trigger policy actions to manage access and affordability is calculated to total approximately $880 million per year for new drugs. Read more

2 WHO Updates List of Essential Medicines to Include Key Cancer, Diabetes Treatments (WHO)

Among the drugs added are pembrolizumab for metastatic cervical cancer, metastatic colorectal cancer, and metastatic non-small cell lung cancer. GLP-1 receptor agonists, semaglutide, dulaglutide, liraglutide, and tirzepatide, to treat diabetes were also added to the Essential Medicines List. Read more

3 Prioritizing the Primary Prevention of Heart Failure (The Lancet)

The first of a series of 3 papers reviews the contemporary global epidemiology and pathophysiology of heart failure and proposes a comprehensive framework for screening to assess heart failure risk and detect pre-heart failure. Read more

4 A Patient-Centered Pathway for Biomedical Innovation and Access (Health Affairs Forefront)

A unified data ecosystem between the US Food and Drug Administration and the Centers for Medicare and Medicaid Services (CMS) is essential, and linking cost and outcomes data would allow CMS to refine payment policies and give manufacturers a clearer, evidence-based path toward approval and coverage that builds on collaborations while meeting post-approval commitments. Read more

5 Real-World Efficacy and Safety of Trastuzumab Deruxtecan Versus Trastuzumab Emtansine and Tucatinib as Second-Line and Third-Line Treatments for HER2-Positive Metastatic Breast Cancer: Two Target Trial Emulation Studies (The Lancet Regional Health Europe)

Using the French National Health Data System, researchers emulated trastuzumab deruxtecan (T-DXd) versus trastuzumab emtansine (T-DM1) and T-DXd versus tucatinib, for second- and third-line HER2-positive metastatic breast cancer treatment and found that T-DXd was more effective than T-DM1 as a second-line treatment and tucatinib as a third-line treatment, in line with clinical trial results. Read more

6 Global Inequities in Diabetes Technology and Insulin Access and Glycemic Outcomes (JAMA Network Open)

The cross-sectional study, which collated data regarding the accessibility and reimbursement of diabetes technologies and insulin from 81 centers across 56 countries, determined that there is significant global disparity and an association between glycemic outcomes and the accessibility of diabetes technologies and insulin. Read more

7 Women, Older People, and Black People Less Likely to Receive an SGLT-2 Inhibitor Prescription for Type 2 Diabetes (NICE)

Only 1 in 5 people with atherosclerotic cardiovascular disease (ASCVD) received SGLT-2 prescriptions, and NICE's analysis noted age- and gender-related disparities, with 32% of people aged 50 to 59 with ASCVD receiving prescriptions compared to just 13% of those aged 80-89 years, and men (35%) more likely to receive prescriptions than women (23%) among people with heart failure. Read more

8 The Cost-Effectiveness of Tafenoquine Following Screening With STANDARD™ G6PD Screening for the Treatment of Vivax Malaria in the Brazilian Public Health System (The Lancet Regional Health Americas)

The use of tafenoquine on those who test G6PD normal with a semiquantitative test is a cost-effective strategy for the radical cure of vivax malaria in Brazil, researchers determined, saying the strategy was US$2894 (R$14,934) per disability-adjusted life-year averted compared to current practice, well below the willingness-to-pay threshold. Read more

9 The Potential of Kidney Transplantation to Reduce Mortality From Chronic Kidney Disease: A Global, Cross-Sectional, Modeling Study (The Lancet Global Health)

Countries with higher kidney transplantation rates have lower mortality–prevalence ratios, regardless of gross domestic product per capita, and dialysis does not. As chronic kidney disease worsens and economic factors affect kidney transplantation rates, policy makers need to increase access to safe and ethical transplantation. Read more

10 Patient and Physician Perceptions of Prostate-Specific Antigen Testing Among Black Individuals (JAMA Network Open)

The qualitative study found that primary care practitioners may not value prostate-specific antigen (PSA) testing for prostate cancer early detection or appreciate its role in reducing the risk of prostate cancer–related mortality. The results suggest that patients and practitioners need improved access to accurate and evidence-based information regarding prostate cancer risk and PSA testing among Black men. Read more

Climbing the HEOR Career Ladder: Strategies for Success in an Era of Change

David Thompson, PhD, Rubidoux Research LLC, Manchester-by-the-Sea, MA, USA; **Christopher Blanchette, PhD, MBA**, Clinical, Medical and Regulatory Affairs, Princeton, NJ, USA; **Stephanie Earnshaw, PhD**, Access Strategy Consulting, Pittsboro, NC, USA

Introduction

Since its inception nearly 3 decades ago, ISPOR has been instrumental in furthering its members' training and development in the tools and techniques of health economics and outcomes research (HEOR). ISPOR short courses, educational webinars, and online resources predominantly focus on HEOR methodology, with content ranging from beginner-level fundamentals to advanced topics on the frontiers of innovation. ISPOR's Health Economics and Outcomes Research Competencies Framework—developed to structure professional development needs and identify gaps in current training opportunities—also has a strong methodologic focus.

> The prerequisites for career advancement have evolved beyond just demonstrating increasing levels of methodologic expertise.

Yet, as the field of HEOR has matured and become more established as a profession, the prerequisites for career advancement have evolved beyond just demonstrating increasing levels of methodologic expertise. In biopharma, HEOR teams are now less siloed and more integrated with other functions, such as market access and medical affairs, as value evidence has become table stakes to a product's commercial success. Similarly, in the larger consultancies and contract research organizations (CROs), HEOR is less often positioned as a "boutique" standalone and more frequently integrated into bundled service offerings that include clinical and real-world evidence generation and/or commercialization support.

Artificial intelligence (AI) presents yet another disruptor to traditional HEOR methodologists' career paths, as it transforms how data are analyzed, results interpreted, and content generated across all disciplines. At present, AI is a key enabler to "human-in-the-loop" processes for prototypical HEOR projects like systematic literature reviews and analyses of real-world data sources, which confers a competitive advantage to those with fluency in these tools and techniques. But some see the foreboding specter of full-blown automation of these activities in the future.

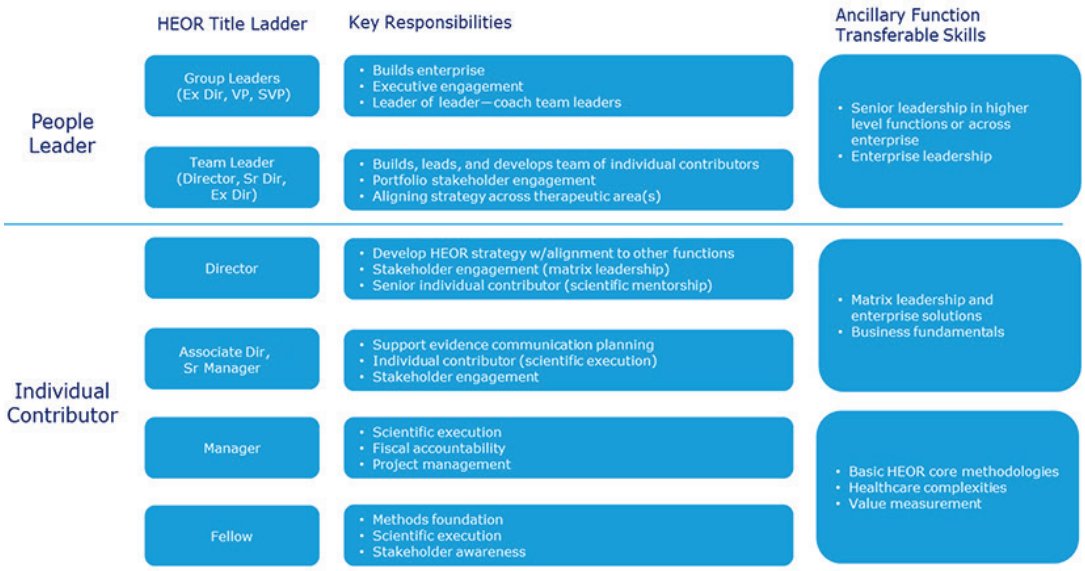
These trends significantly impact career advancement strategies in HEOR. Professionals must now transcend pure methodology, cultivating broader strategic thinking, effective cross-functional communication, and deeper understanding of HEOR's commercial relevance. These uniquely human capabilities become more valuable as AI assumes responsibility for routine research processes. In this article, we build on issues and themes we discussed in a forum session earlier this year at ISPOR 2025 in Montreal. While our focus is on life sciences companies and CROs/HEOR consultancies, much of the discussion applies to careers in government and academia as well, which are similarly experiencing significant disruption and realignment.

Career advancement in life sciences organizations

In biopharma, the value of core training in HEOR is widely accepted as critical to all functional areas. With health economists and outcomes researchers increasingly integrated with other functions, there are opportunities for those with HEOR training to apply these fundamental skills to other critical areas of the business that we typically don't receive training in. Using HEOR training as a fundamental base, we can enhance our skills to include project management, financial acuity, enterprise mindset, and marketing, to name a few, thereby unlocking a rewarding career opportunity across the industry.

As illustrated in the career ladder in **Figure 1**, the first stage at which one may take these skills to other functional areas is immediately after graduate or fellowship training in the fundamentals of HEOR when there is an expectation of

Figure 1. HEOR career progression in life sciences organizations



Ex Dir, Executive Director; VP, Vice President; SVP, Senior Vice President; Sr Dir, Senior Director; Associate Dir, Associate Director; Sr Manager, Senior Manager.

methodologic competency. The second stage is after a few years of an entry-level HEOR role where one has gained more widespread experience in the application of these methods and where one has broadened their knowledge base as individuals working in cross-functional teams to solve business challenges that touch across multiple functions. The final stage is when one has leadership responsibilities and has moved away from applying the methods. In these roles, HEOR fundamentals can enhance the broader higher-level functions and be utilized in other functions across the organization. It is also important to note that experience in the ancillary functions can be valuable to bring back to the core HEOR studies.

Continued professional development as the environment changes is critical to one's success in industry. As AI and more data science technologies continue to advance, HEOR professionals will need to learn how to harness these technologies to deliver studies, models, and insights more efficiently to meet the needs of the business. This should not be seen as a threat but rather a tool to enable greater impact of our work. AI may be thought of as similar to the invention and increased use of computer hardware/software back in the 1970s, 1980s, and 1990s. One would hardly argue that the advances in computer processing and statistical software have inhibited our advancement in HEOR, but rather they have made us more efficient and productive. Think about it. It would be hard to do much of our work with an abacus. Similarly, embracing AI assistance with coding, writing, model development, etc., will allow us to deliver faster and more targeted results that enable us to understand and interpret for stakeholders, customers, and executives.

Career advancement in contract research organizations and consulting firms

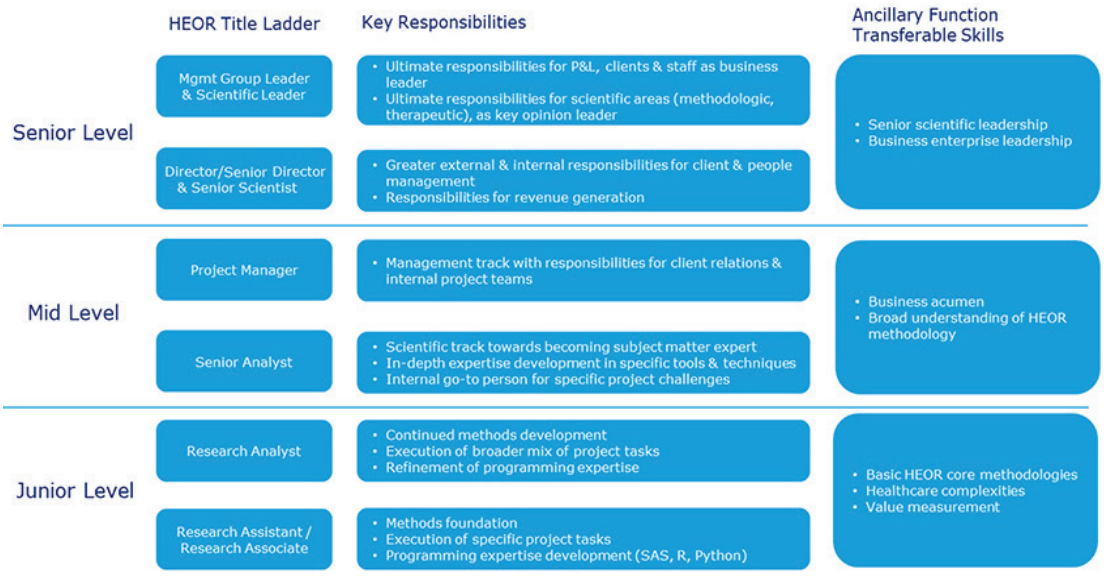
For those in the consulting world, supporting the pharmaceutical industry has been the primary focus for revenue generation. Students from highly respected health system and pharmacy administration, pharmaceutical systems and policy, and other highly mathematical/statistical programs would step onto the career ladder at the junior level, as shown in **Figure 2**. Often, team members within these organizations grow their careers by becoming experts in a very specific scientific method (eg, evidence synthesis, comparative effectiveness, economic modeling, analysis of real-world data). Within these organizations, team members would move up in their careers by transitioning from the "doer" role to the "project manager" role. In some situations, team members can choose a more technical path to be an expert in a very specific method, on a track that will develop their subject matter expertise towards ultimately becoming a key opinion leader in their chosen specialization.

> Professionals must now transcend pure methodology, cultivating broader strategic thinking, effective cross-functional communication, and deeper understanding of HEOR's commercial relevance.

However, as the field has evolved, knowing the science has not been enough. There is a critical need to embed HEOR earlier in the product development life cycle as well as in the middle and towards the end of the life cycle. Having the full product life-cycle picture enables individuals to answer the "so what", "how do we align the outcomes with other work and with clinical/market access messaging", and "how to successfully communicate the work" questions. Being strategic and understanding these aspects are of the utmost importance. These are service offerings that are more often best provided by the larger consultancies and CROs or by individuals who have the full product life-cycle picture.

In today's financially constrained market, embracing the use of AI and understanding the application of HEOR to health interventions other than pharmaceuticals will also be critical. Specifically, performing HEOR more broadly and efficiently will be the key to success. AI will enable consultancies to create efficiencies, but AI may not be as capable on the strategy front. As the importance of HEOR grows, an expanded and strategic mindset is becoming essential to providing guidance around what we do, how we apply HEOR, and how we communicate our findings.

Figure 2. HEOR career progression in CROs and consultancies



Conclusions and implications

The HEOR profession stands at a pivotal juncture

where methodologic expertise is necessary but not sufficient for career advancement. HEOR professionals need to expand not just their skillset, but also their mindset—to move beyond technical proficiency to encompass business acumen, cross-functional collaboration, and strategic thinking.

> For HEOR professionals navigating this evolving landscape, the implications are clear: career success requires intentional skill diversification and strategic thinking.

Within pharma, the integration of HEOR teams with market access, medical affairs, and other commercial functions has fundamentally altered the career path, requiring professionals to demonstrate not only what the research shows, but how it aligns with broader business objectives and product life-cycle strategies. The bundling of HEOR with broader clinical and commercialization services within the larger CROs and consultancies requires understanding how HEOR contributes to the overall client picture.

The emergence of AI as both an enabler and potential disruptor presents a significant challenge demanding proactive adaptation instead of defensive resistance. While AI increasingly automates routine analytical tasks—from systematic literature reviews to real-world data analysis—it simultaneously creates opportunities for HEOR professionals to focus on higher-value activities such as strategic interpretation, stakeholder communication, and business integration. Those who successfully harness AI as a productivity tool while continuing to develop their distinctly human capabilities in strategic thinking and cross-functional leadership will find themselves better positioned for career advancement than those who remain confined to purely methodologic roles.

For HEOR professionals navigating this evolving landscape, the implications are clear: career success requires intentional skill diversification and strategic thinking. Whether in biopharma, consulting, or other sectors, professionals must actively seek opportunities to broaden their experience beyond core HEOR methodology, developing competencies in commercialization, financial understanding, and strategic communication. ISPOR is already evolving its training and development offerings to reflect these changing requirements, ensuring that the next generation of HEOR professionals is equipped not only with methodologic rigor but also with the business savvy and flexibility necessary to thrive in an increasingly integrated and AI-augmented healthcare landscape.

ISPOR Stimulates Discussion About US Drug Price Negotiations in Inaugural Health Policy Retreat

Beth Fand Incollingo

ISPOR's first-ever health policy retreat explored America's Inflation Reduction Act (IRA) and the possibility that the United States will introduce most-favored-nation (MFN) drug pricing, highlighting how the measures are expected to affect payers, patients, and innovators.

An exclusive, invitation-only event for 30 of ISPOR's corporate sponsors and selected health policy experts, the Health Policy Retreat 2025 was held June 14 at the Fairmount Georgetown in Washington, DC. Its intimate format was designed to enable both presenters and an audience of consultants, payers, patients, academics, and biopharmaceutical experts to speak freely.

> "As we look to the future, it is absolutely vital that we think about how to use the rigor, evidence, and story of the science to inform health policy decision making."

"It's an opportunity to ask the questions that are legitimately keeping you up at night," Rob Abbott, ISPOR's CEO and executive director, told the group.

Abbott said the gathering was the first of many ISPOR will host to discuss health policy issues.

"What we're doing today is something that has been an aspiration for our Society for quite some time because ISPOR has always been and, importantly, always will be a steward of the science that underpins the profession of health economics and outcomes research (HEOR)," he said. "As we look to the future, it is absolutely vital that we think about how to use the rigor, evidence, and story of the science to inform health policy decision making."

The retreat focused on the specifics of 2 policies: the Medicare Drug Price Negotiation Program and an executive order signed in May 2025 by President Donald Trump. The Medicare Drug Price Negotiation Program, created under the IRA of 2022, aims to make selected high-cost prescription medications more affordable for Medicare beneficiaries by allowing the Centers for Medicare & Medicaid Services (CMS) to negotiate prices with manufacturers. The executive order calls for manufacturers to lower drug prices to match the lowest amounts charged for the same medications in countries that are members of the Organisation for Economic Co-operation and Development and have per capita gross domestic product (GDP) numbers totaling at least 60% of America's GDP.

In a keynote address, Kristi Martin, MS, MPA, healthcare director of the Camber Collective and a former leader with CMS, highlighted issues related to the measures that will likely be a focus for HEOR experts. Adding to that discussion in shorter talks were Sean Sullivan, PhD, a professor of pharmacy at the University of Washington; Stacie Dusetzina, PhD, an associate professor of health policy at Vanderbilt University Medical Center; and John O'Brien, PharmD, MPH, president and CEO of the National Pharmaceutical Council.

Martin said that the changes stimulated by the IRA, including negotiated prices for selected high-cost drugs, a $2000 annual out-of-pocket cap on prescription medicine costs for beneficiaries, and a $35 limit on insulin prescription prices, constitute "the largest changes to the Part D program we've seen since its inception in 2003," some of which will also affect Medicare Part B. The combined effects of the reforms are not yet fully clear, the speakers said, and adjustments may be needed.

They highlighted a number of questions for the HEOR community to consider:

- What is the fairest way to determine which drugs are eligible for price negotiation?
- How will commercial insurers devise prices for drugs that have Medicare-negotiated values?
- Is it fair that some drug types must be on the market longer than others before they can be considered for Medicare price negotiation, or does that discourage pharmaceutical companies from developing certain kinds of treatments?
- Does negotiation of drug prices disincentivize manufacturers from conducting postmarket research on those treatments or seeking additional indications?
- Should the United States establish a broader health technology assessment mechanism, or might a different system be needed, given the later life cycle of the drugs being assessed?
- How can the price negotiation process be kept transparent?

In sorting all that out, "we can really focus on the opportunities by thinking about how to engage in a dialogue that moves the ball forward," Martin said.

Also during the day, a panel discussion titled "International Reference Pricing—What Does the Future Hold?" was moderated by Peter Neumann, ScD, director of the Center for the Evaluation of Risk in Health at Tufts Medical Center, and Laura Pizzi, PharmD, MPH, chief science officer at ISPOR. Panelists were Anthony Barisano, PharmD, vice president for global HEOR at Bristol-Myers Squibb; Katie Keith, JD, MPH, a director of the O'Neill Institute for National and Global Health Law; Sean Dickson, JD, MPH, a senior vice president with AHIP; and patient advocate Sue Peschin, MHS, president and CEO of the Alliance for Aging Research.

Discussion points included:

- The enforceability of the executive order mandating MFN pricing
- How tariffs might affect that pricing
- Whether it's a good strategy for the United States to "import" drug prices based on other countries' conceptions of value
- The possibility that MFN pricing will induce manufacturers to pull out of smaller foreign markets
- The potential for pharmaceutical companies to profit even if drugs are priced below their value

Because considerations about value are a part of all healthcare policy decisions, Pizzi queried the group about how ISPOR can help define the term.

"Many of us will have different views, but there's a Venn diagram somewhere," Barisano said. "Start by asking multiple stakeholders to choose the values they agree are worth including in pricing discussions—values that will ultimately give back to patients."

Beth Fand Incollingo is a freelance writer who reports on scientific, medical, and university issues.

ISPOR Conferences and Events

Thank you to the sponsors of the ISPOR Real-World Evidence Summit 2025: *Through the Lens of Asia Pacific*

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ISPOR Europe 2025 | 9-12 November
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Powering Value and Access Through Patient-Centered Collaboration

Through cutting-edge research, dynamic discussions, and real-world case studies, ISPOR Europe 2025 is set to highlight the latest advancements in HEOR that enhance patient-centered innovation, advance healthcare access, and drive better outcomes for all.

Program Highlights

New **Patient-Centered Evidence Track!**
This new track complements ISPOR's Patient-Centered Research interest area and is dedicated to elevating patient engagement by recognizing and leveraging patients' expertise, lived experiences, and knowledge to drive meaningful advancements in HEOR.

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- **Pragmatic Trials : Bridging Research and Real-World Care**
- **RWE in European Healthcare Decision Making : What's in it for Patients?**

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ISPOR Conferences and Events

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Philadelphia Convention Center | Philadelphia, PA, USA

Healthcare stakeholders will convene at ISPOR 2026, the leading global conference for HEOR, for discussion and dissemination of the latest trends in healthcare.

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Session and HEOR Impact Case Study Abstract Submission Closes	**December 12**
Research Abstract Submission Opens	**October 31**
Research Abstract Submission Closes	**January 9, 2026**

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ISPOR Education

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Upcoming In-Person Courses:

9-12 November | Glasgow, Scotland, UK

11 courses to choose from on 9 November, including:

- Introduction to Applied Generative AI for HEOR
- Prompt Engineering for HEOR: Practical Skills and Use Cases for HEOR Professionals
- Reimbursement Systems for Pharmaceuticals in Europe
- Using LLMs to Simplify Real-World Evidence Research
- Valuation of Innovative Drugs

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December 2 | 11:00AM – 12:00PM EST

HEOR Meets Investing: Why Are Banks and VCs Collaborating With Health Economists?

In recent years, there has been a growing trend of HEOR professionals collaborating with investment banks and venture capitalists (VC). Why are banks and VCs thinking about health economics? Can health economics inform investment decisions? Can HEOR methods and thinking improve return on investment for life science investors? This webinar, brought to you by ISPOR, will explore these questions and more.

December 3 | 10:00AM – 11:00AM EST

The Application of AI in COA Research: Opportunities and Challenge

The rise of artificial intelligence (AI) is transforming research across disciplines, bringing both enthusiasm and concern, including within the context of clinical outcome assessment (COA) research. This webinar, brought to you by the ISPOR Clinical Outcome Assessment Special Interest Group, will explore the emerging applications, potential challenges, and critical considerations of using AI in COA research.

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HEOR ACROSS THE GLOBE

ASIA PACIFIC

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

Ripples That Reached Far Shores: HEOR Conferences in the Asia-Pacific Region

Paula Lorgelly, PhD, University of Auckland, New Zealand

Unuhia te rito o te harakeke, kei hea te kōmako e kō?
Ui mai ki ahau, "He aha te mea nui o te Ao?"
Māku e kī atu,
"He tangata, he tangata, he tangata."

If you remove the central shoot of the flaxbush, where will the bellbird find rest?
If you were to ask me, "What is the most important thing in the world?"
I would reply,
"It is people, it is people, it is people."

This is a Māori proverb (a whakataukī) from the indigenous people of Aotearoa New Zealand. It uses a flax bush (a common New Zealand plant) as a metaphor for a large family unit, a whānau. Flax is used by weavers, but they only take the outer leaves; removing the middle stops growth and flowers, and without flowers there isn't nectar for the bellbird. The bellbird would fly between land and sea, searching for a perch and food. Similarly, if the whānau (flax) ceases to produce and nurture children (new leaves) it will die. The proverb shows the importance of the whānau unit for nurturing people.

As this issue of *Value & Outcomes Spotlight* highlights the HEOR Workforce, I thought I'd adapt the metaphor, such that the flax bush syllabizes the recent conferences in the Asia Pacific region. Our outer leaves might have been cut by COVID, but we are back thriving in the global community and showing the world our flowers and they have come to drink our nectar.

The first event that kicked off the conference season in the region was the 16th International Health Economics Association (IHEA) Congress in Bali, Indonesia in July. I was honored to be the scientific program chair of this meeting; although it was a demanding role as we broke records with the number of abstracts submitted, and we unfortunately couldn't accept them all. We had more than 1500 delegates—the second largest IHEA congress ever, and notably 38% of attendees were from the Asia region. It was intellectually rich, stimulating, and inspiring, and importantly showcased health economics in the region.

In August, Singapore hosted the 12th HTAsiaLink Conference. HTAsiaLink supports knowledge transfer and exchange across the Asia Pacific region, building the capacity for health technology assessment (HTA) and promoting the integration of HTA into decision making. There was a full program of science and debate that looked at diversity and equity, new developments in artificial intelligence and innovative technologies, and the role of HTA in achieving value-based healthcare. It was a close-knit, members-only event, which saw the launch of the HTAsiaLink Strategic Plan and the celebration of the 10th anniversary of Singapore's Agency for Care Effectiveness.

We closed off the season with the ISPOR Real-World Evidence Summit in Tokyo in late September. I was a member of the program committee and again the quality of the abstracts submitted was excellent. Testament to the growth in scientific rigor in the region. Over 2 days, nearly 700 attendees discussed a range of issues with respect to harnessing and improving real-world evidence in the region to support the achievement of healthcare goals and growth strategies. Global experts, regional decision makers, and industry leaders discussed research and trends with respect to our regional landscape. Networking opportunities were aplenty, particularly among the different ISPOR Asia Pacific Chapters.

In 2025, the Asia Pacific region has showcased our beautiful cultures, stunning scenery, and culinary delights, while the HEOR workforce has networked, engaged, and established new collaborations across our scientific communities.

It is the people, it is the people, it is the people that make HEOR amazing.

Acknowledgment
I wish to acknowledge Metge and Jones He Taonga Tuku Iho no Ngā Tupuna. Maori proverbial sayings—a literary treasure, New Zealand Studies, July 1995 for providing an explanation of the whakataukī.

HEOR ACROSS THE GLOBE

EASTERN EUROPE, MIDDLE EAST, AND AFRICA

Section Editor: **Bertalan Németh, PhD**, Budapest, Hungary

New Developments and Regulations in Poland

Maciej Niewada, MD, PhD, Warsaw University of Technology, Poland

The current situation in Poland, which is indeed quite interesting, includes many new regulatory changes in the field of pricing and reimbursement of medicines, as well as HTA. Polish society is expecting a new amendment to the Reimbursement Regulation. However, the changes involve local, country-specific arrangements and may not be particularly relevant to share more broadly.

On the other hand, Poland does have some developments of greater scientific interest. The Polish HTA Agency has begun work on multiple criteria decision analysis for rare diseases. The process is still in its early stages, but we hope to have a more fully developed framework early next year.

In addition, the Polish HTA Agency is involved in one of the first Joint Clinical Assessment processes. While this is not yet mature enough to provide substantial insights, we expect that by the end of Q4 some initial experiences will be communicated.

ISPOR's Bulgaria Chapter in Action

Guenka Petrova, DSc, PhD, Medical University of Sofia, Bulgaria

The Bulgarian ISPOR Chapter will participate at the 9th pharmaceutical congress of the Bulgarian Scientific Pharmaceutical Association. The congress motto is PharmaceuticAI Science Revolution. There will be a special session devoted to artificial intelligence (AI) application in pharmacology. Members of the Bulgarian ISPOR Chapter will present their work in the field of use of AI for evidence synthesis, real-world data collection and analysis, and practical application in the field of pharmacology.

The congress will also touch the global problems related to rational drug design, chemotherapy, targeted antineoplastic therapy and drug resistance, clinical pharmacology, hospital pharmacology, pharmacokinetics and biopharmaceutics, and others.

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

RESEARCH ROUNDUP

Section Editor: **Aakash Bipin Gandhi, BPharm, PhD**, Sanofi, Cambridge, MA, USA

Competencies for professionals in health economics and outcomes research: the ISPOR health economics and outcomes research competencies framework.

Pizzi LT, Onukwugha E, Corey R, Albarmawi H, Murray J. *Value Health*. 2020;23(9):1120-1127.

Summary

This article describes the development of the ISPOR Health Economics and Outcomes Research (HEOR) Competencies Framework, created to address the growing global demand for qualified HEOR professionals. Through a collaborative process involving ISPOR's Institutional Council and Faculty Advisor Council, the framework identified 41 essential competencies for HEOR professionals organized into 13 topic domains: (1) Business Management, (2) Career Development, (3) Communication & Influence, (4) Economic Evaluation, (5) Health Policy & Regulatory, (6) Health Service Delivery & Process of Care, (7) Study Approaches, (8) Patient-Centered Research, (9) Methodological & Statistical Research, (10) Clinical Outcomes, (11) Health Technology Assessment, (12) Epidemiology & Public Health, and (13) Organizational Practices. The development methodology combined expert input from council members, natural language processing of HEOR job postings, qualitative assessment of data from focused workgroup, and quantitative data from 3 distinct surveys targeting general members, faculty, and students.

Relevance

The resulting framework serves multiple purposes, including guiding curriculum development for academia, informing program design for industry fellowships, structuring educational offerings by ISPOR, and providing standards for evaluating job candidates. As the field of HEOR evolves, the framework will require periodic updates to maintain relevance and comprehensiveness, with potential future specializations for different HEOR paths.

Predictors of annual base salary for health economics, outcomes research, and market access professionals in the biopharmaceutical industry.

Ghosh S, Rascati KL, Shah A, Peeples P. *J Manag Care & Spec Pharm*. 2019;25(12):1328-1333.

Summary

The study by Ghosh et al examined key factors influencing compensation among health economics, outcomes research, and market access professionals in the biopharmaceutical sector using data from the HealthEconomics.com 2017 Global Salary Survey. An analysis of 385 respondents (228 US based and 157 non-US based) revealed no statistically significant gender-based salary differences after controlling for relevant confounders. The median annual base salary in the United States was $172,500 (male) versus $162,500 (female). This was higher than the median non-US annual base salary of $92,500 (both genders). Factors found to be associated with higher compensation across all regions included age over 40 years, employment in biopharmaceutical companies, advanced degrees (PhD, PharmD, or MD), senior job titles (director levels), having budget management authority, having hiring authority, and working in organizations with more than 5000 employees.

Relevance

This research addresses the critical gap surrounding HEOR and Market Access compensation data, providing essential insights for employers and employees in terms of competitive pay practices. Despite the field's growing importance in healthcare industries, transparency around salary information remains scarce. By analyzing compensation structures across this cross-functional discipline with its diverse talent pool, the study enables data-driven decisions for recruitment and retention strategies for employers and also provides essential insights for professionals pursuing employment opportunities in the field.

Note from the Section Editor: *Views, thoughts, and opinions expressed in this section are my own and not those of any organization, committee, group, or individual that I am affiliated with.*

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.

Emerging Trends: Artificial Intelligence in HTA

Artificial intelligence (AI) is increasingly being recognized as a tool with the potential to reshape health technology assessment (HTA). In recent months, several HTA agencies have begun to clarify how AI may support evidence generation, evaluation, and decision making.

The Canadian Drug Agency (formerly CADTH) was among the first HTA organizations globally to explore the implications of AI. In 2018, it published scoping reviews on AI's potential applications in clinical research and evidence synthesis, highlighting how machine learning could reshape systematic review methods and data appraisal. At the time, this was largely a forward-looking exercise, but it reflected a growing recognition that AI would soon challenge traditional HTA processes. What the Canadian Drug Agency anticipated then is increasingly becoming reality today.

The National Institute for Health and Care Excellence (NICE) in England has since taken a leading role in the implementation of AI into routine HTA processes. In August 2024, NICE issued a position statement on the use of AI in evidence generation, outlining both opportunities and risks. To promote methodological transparency, it introduced the use of CHEERS-AI, a new reporting standard for economic evaluations of health interventions using AI technologies. It has also piloted its Early Value Assessment process with an AI triage tool for skin lesions. Beyond evaluations of specific technologies, NICE's HTA Lab is exploring how generative AI can assist in economic modeling and evidence assessment.

Other European agencies are also active in considering how AI can be integrated into HTA methods. The independent Institute for Quality and Efficiency in Health Care (IQWiG) in Germany has examined how AI could support systematic reviews and evidence retrieval, hosting a meeting on AI tools for information retrieval in 2024. According to a poster presented by IQWiG at ISPOR Europe 2024, they were the first agency to test classifiers for screening in literature reviews. The High Authority of Health (HAS) in France developed an assessment grid for medical devices embedding AI, and HAS continues to link its evaluations with national health data and AI strategies.

At a broader level, professional organizations are also actively engaging in this topic. ISPOR has convened a dedicated working group on AI in HTA and health economics and outcomes research (HEOR), tasked with developing methodological guidance on the responsible use of AI in evidence generation, economic modeling, and policy support. The ISPOR report "Generative Artificial Intelligence for Health Technology Assessment: Opportunities, Challenges, and Policy Considerations" explores how generative AI and large language models (LLMs) can transform the HTA process. It highlights key applications, such as accelerating systematic literature reviews, analyzing real-world evidence, and aiding in health economic modeling. While acknowledging these significant opportunities, the report also outlines critical challenges, including the risk of AI "hallucinations," data privacy concerns, and the potential for algorithmic bias. Ultimately, the report emphasizes that AI should serve as a supportive tool for HTA professionals, not a replacement, and calls for robust ethical and regulatory frameworks to ensure its responsible and effective implementation.

While regulatory frameworks for integrating AI into HTA methods are still being developed, the advancements made to date by NICE, CDA-AMC, IQWiG, HAS, and ISPOR undoubtedly illustrate that AI is already shaping both health interventions as well as HTA methods to evaluate them. A broader debate on validation, standards, adoption, transparency, and patient engagement in the context of the appropriate use of AI in HTA will help to inform the best way forward.

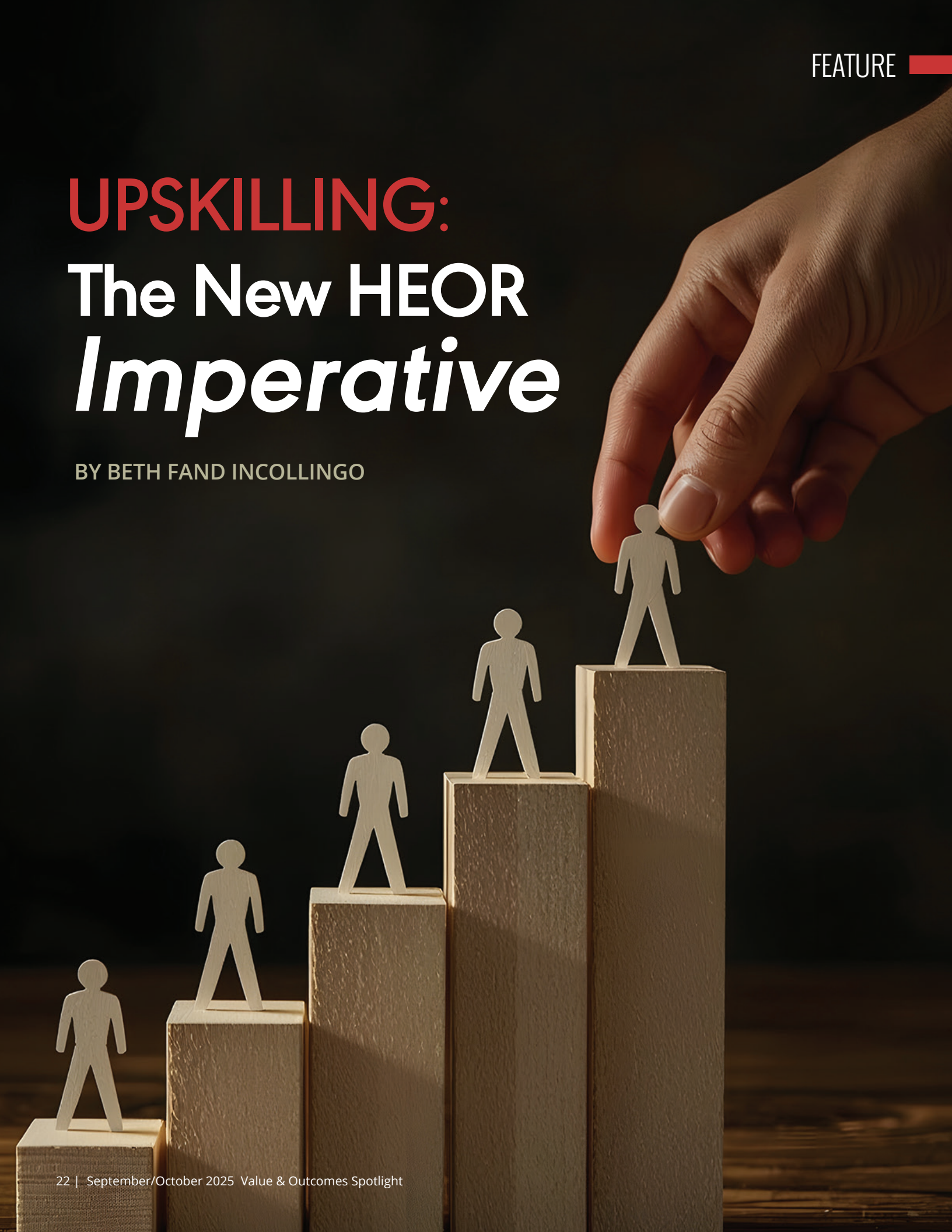
Editor's Note

The Editors of *Value in Health* have partnered with the PhRMA Foundation on a special Call for Papers focusing on "Artificial Intelligence in Health Economics and Outcomes Research." Led by Guest Editors Jaime Caro, PhD, MDCM, FRCPC; Jagpreet Chhatwal, PhD; and Rachael Fleurence, PhD, MSc, this themed section will explore the role of AI technologies, with a special emphasis on the novel capabilities and applications of generative AI.

The PhRMA Foundation will recognize select papers from early career researchers for a PhRMA Foundation Challenge Award of $5000, which will be awarded to the first author of the award-winning papers. The complete collection of these AI-focused papers will be published in the November 2025 issue of *Value in Health*. Watch the *Value in Health* website for the forthcoming publication!

UPSKILLING: The New HEOR *Imperative*

BY BETH FAND INCOLLINGO



Over the past 2 decades, health economics and outcomes research (HEOR) has evolved into a cornerstone of healthcare decision making. Key trends include the rise of real-world evidence (RWE) to complement clinical trials, a shift toward value-based care, and greater emphasis on patient-centered outcomes. Through this time, HEOR has gained global influence in health technology assessments (HTA) and policy shaping. A new era of rapid change is upon us—driven by more sophisticated methodologies incorporating artificial intelligence (AI) and advanced modeling techniques.

That's the way Louise Parmenter, PhD, global head of scientific services, real-world evidence at IQVIA, sums up the changes affecting experts across the HEOR workforce.

In addition to the emerging use of AI to streamline tasks in a variety of settings, HEOR is being reshaped by regulatory and policy changes. The most notable include the European Union's new joint clinical drug assessment process and the Centers for Medicare and Medicaid Services' final guidance for drug price negotiation.[1] There are RWE policy developments neatly summarized by the RWE Alliance. At the same time, HEOR professionals are being asked to work across diverse therapeutic technologies, disease areas, and health systems. As HEOR professionals strive to keep up, they're finding the opportunities to contribute have changed.

The changes are affecting everyone from health economists and epidemiologists to psychometricians and clinicians. "In my whole career—which is decades, not years—this has probably been the time of the most change," said Parmenter, who is based in the United Kingdom. "That makes things interesting but presents challenges. So, I'm mindful about that with the workforce I lead."

With so many changes afoot, strengthening crucial skills is paramount, and there are many ways for professionals to do that, including through collaboration between academic and private organizations, Parmenter said. She added that IQVIA, a leading global provider of clinical research services, commercial insights, and healthcare intelligence to the life sciences and healthcare industries, offers training to help its workforce stay up to date. HEOR professionals can also learn in courses offered by professional organizations such as ISPOR, which provides educational programs and Professional Development Resources.

"In addition to new regulations and guidelines, there's a huge pace of acceleration of methods, approaches, scientific innovation, and evolution," Parmenter said. "Employees need to stay current on the best methods for conducting research and forward thinking on how AI will evolve their roles."

Constant Change Is Here to Stay

Now that the Great Resignation[2] has subsided, Parmenter has seen a new stability in how the HEOR workforce is distributed.

During the pandemic, "HEOR experts moved around a lot," she said. "Now, much of that has settled, with levels of movement that are slightly below what we'd expect as an average." Unfortunately, there may be fewer employment opportunities than there were 10 or 15 years ago, when the HEOR field experienced a boom,[3] said Christopher Blanchette, PhD, MBA, vice president for clinical data science and evidence at Novo Nordisk.

While Parmenter is seeing demand for HEOR expertise from countries with HTA bodies or government-run copay systems,[4] Blanchette has noticed a trend toward layoffs in the pharmaceutical industry.[5] Company-wide layoffs are often responses to drugs going off patent, he said, but cutbacks that specifically target HEOR functions highlight the need for such groups to better measure and communicate how they're meeting stakeholders' needs.

"When HEOR groups become sizeable, companies sometimes question their return on investment, and, unlike with sales departments, it's hard to pinpoint that," said Blanchette, who oversees teams in the United States and Mexico. "You need people in HEOR to grow into strong leaders who communicate that, and then they need to empower the people under them to be more effective at communicating and delivering that message across the organization."

"In addition to new regulations and guidelines, there's a huge pace of acceleration of methods, approaches, scientific innovation, and evolution. Employees need to stay current on the best methods for conducting research and forward thinking on how AI will evolve their roles."

– Louise Parmenter, PhD

Blanchette added that tariffs and recent cuts to health-related regulatory bodies by the US government[6] may have resulted in some job loss across HEOR and could lead to more, due to a cascade that will first affect pharmaceutical companies and then reach consulting groups, researchers, academics, investors, and biotechnical startups.

Turbulence across the field is just as much a factor for those already engaged in HEOR work. For instance, Parmenter said, since the European Union began conducting HTAs as a unified entity in January 2025,[7] HEOR workers have been called upon to enter the process earlier, learn new ways to present clinical information, and collaborate with the regulatory bodies of multiple countries at once.

In addition, the work of HEOR professionals must incorporate new guidelines released by many countries around the use of RWE,[8] and must account for a related initiative — the European Medicines Agency's Data Analysis and Real World Interrogation Network, or Darwin EU, which provides compiled data that can be used by the EMA and national competent authorities in the European medicines regulatory network whenever needed throughout the lifecycle of a medicinal product.

"Decision makers now have direct access to safety and effectiveness data, and that's a change that helps with efficiency," Parmenter said of Darwin. "Nevertheless, there will continue to be strong demand for HEOR expertise, because the whole field has become more complex."

You Can't Spell "Transformation" Without "AI"
As perhaps the field's biggest disruptor, AI presents the workforce with both challenges and solutions. The key is in learning to understand and apply the technology.

Rachael Fleurence, PhD, who leads global evidence and AI solutions initiatives at Value Analytics Labs after a public health career that included a post at the National Institutes of Health, called the introduction of the technology "a watershed moment."

"Compared with 30 years of incremental changes," she said, the technology is "the most disruptive we've had for a long time."

Fleurence is excited about AI's ability to make HEOR projects not only more comprehensive and effective, but also more streamlined, so that workers will have more time for other tasks.[9]

> "You need people in HEOR to grow into strong leaders and they need to empower the people under them to be more effective at communicating and delivering that message across the organization."
>
> – Christopher Blanchette, PhD, MBA

Currently, she's overseeing 2 literature reviews that are employing AI to assist with screening, data extraction, and report writing. Her company is also testing AI's ability to generate economic outcomes decision-making models.

In addition, Value Analytics Labs has developed ValueGen.AI, a platform that can generate automated, disease-specific landscape reviews for pharmaceutical and medical technology companies.[10] The reviews summarize the market, competitors, HTA decisions, demographics, epidemiology, and the pipeline—with information being gathered much more quickly than in the past.

"Before, you'd have folks on the team going through thousands of abstracts manually to see if they should be included in a review, but AI is able to rank the abstracts so humans can see which should most likely be included first and which should most likely be excluded," Fleurence said. "You can really save months of work."

> "There will continue to be strong demand for HEOR expertise, because the whole field has become more complex."
>
> – Louise Parmenter, PhD

IQVIA has also agentified its literature review process. In addition, employees are using AI to do medical and scientific writing. The potential benefits are huge, including making it easier for subject-matter experts across a host of countries to partner in writing clinical study reports or value dossiers, speeding time to delivery and improving quality. But it does not end there; AI makes what was impossible for humans possible, with the potential to manage scale and pace of change in ways previously unimaginable.

On the horizon, Fleurence envisions living HTA assessments, which instead of being compiled and shelved will be systematically updated via AI as new drugs enter the market.

Both companies are being cautious, however, to ensure that there's always a human in the loop to guide AI and gauge its success. "We're still doing a lot of human validation, because these are new tools and their output needs to be scientifically valid," Fleurence said. "The exciting part is that it does seem like generative AI is quite helpful."

Professional Development Is a Necessity
So, how can the HEOR workforce keep up?

The key, Parmenter said, is professional development. "With the environment changing from the regulatory, methods, and technology standpoints, none of us can be complacent," she said. "We all need to see ourselves as being on a journey of continuous learning."

That's why every HEOR professional seems to have a voluminous reading list, she said, which is helpful but can make it difficult to know what to tackle first. "If you just look on the internet, you can access a lot of technological training, but it can be a bit overwhelming," she said. "What's interesting is that

you can actually use AI to summarize the information in a more digestible way."

Parmenter recommended that HEOR professionals also increase their knowledge by collaborating with colleagues. At conferences and through organizations like ISPOR, the International Society for Pharmacoepidemiology (ISPE), and the Drug Information Association (DIA), she said, IQVIA encourages team members to teach courses alongside colleagues from academia, industry, service-providing organizations, and regulators, as well as attending classes.

> "Compared with 30 years of incremental changes, [AI is] the most disruptive we've had for a long time."
>
> – Rachael Fleurence, PhD

"One of the nicest ways to advance thinking and methods is this kind of collaboration across institutions, bringing those different perspectives together," she said. "It can accelerate learning and lead to some of the best papers—the type that end up being cited again and again."

After educational events, IQVIA asks those who participated to spread their new knowledge throughout the company via internal symposiums. Likewise, employees who publish their findings offer seminars to IQVIA's teams.

"In a monthly continuing scientific education series, speakers tell us about the latest analytic methods or approaches," she said, "and we also have centers of excellence that promote best practices in changing regulatory and payer environments. The only way to stay abreast of all that is to keep learning and to participate in some way, whether that means submitting a poster to a conference or finding a mentor within our organization."

IQVIA also circulates a twice-yearly newsletter to inform employees about new regulations and guidelines, and it trains employees to use AI through a program that awards color-coded belts for increasing levels of expertise.

AI adoption is being actively encouraged through active soliciting of AI use cases, pilots, and a formal roll-out and change management process to accelerate transformation.

In the larger HEOR community, Blanchette hopes to eventually see collaborations between the nonprofit and academic sectors to create what he believes is missing in professional development: high-level courses that teach senior HEOR professionals "the soft skills of business and communication, such as how to interact with boards and how to work through mergers and acquisitions."

For those just starting out, technical skills should be the primary focus, and that's why university students should increase their value by doing everything they can to absorb AI techniques, added Blanchette, a former associate professor at the University of North Carolina at Charlotte.
Parmenter cautioned, though, that even the newest job candidates might need some professional development. "We have quite a few internship schemes that we offer in India, Portugal, the United Kingdom, the United States, and elsewhere," she said, "which enable us to work with local universities to understand what individuals have learned and then bring in a cohort of employees to give them the extra training they might need."

Growing Pains for Pharmaceutical Teams

Many of the supports available to the HEOR workforce involve collaboration, but is it possible for that effort to go too far?

There has been some debate about recent decisions by pharmaceutical companies to disband their HEOR groups and embed members across other departments.

Parmenter thinks the trend could be a response to HEOR experts being needed earlier and more consistently throughout the drug-development process now that RWE is so often used to supplement phased clinical trials.

But Scott D. Ramsey, MD, PhD, director of the Hutchinson Institute for Cancer Outcomes Research at Fred Hutch Cancer Center, has expressed concern about the reshuffling because it has led to the layoffs of HEOR department heads, depleting organizational expertise.[11]

> "We all need to see ourselves as being on a journey of continuous learning."
>
> – Louise Parmenter, PhD

"It's a paradox," Parmenter said. "The benefit of having HEOR experts together is that you get the intensity of the brain trust around the discipline and the career paths of individuals, with built-in mentoring and a clear path up the career ladder. On the other hand, people can be a little siloed in that model and disconnected from applying their scientific thinking to the business."

She suggested that embedded HEOR professionals who "feel isolated from their academic homes" get involved with

professional bodies like ISPOR, which "can offer the ability to remain connected while remaining close to the heart of the business you've been hired to support."

No matter how HEOR professionals are deployed, Blanchette added, they are most likely to be successful if they focus on what is needed by the stakeholders they serve and work cross-functionally within their organizations to deliver it.

"Even though you're contributing through HEOR," he said, "you should think about how your work impacts market access, medical messaging, or commercial value."

Changing Work Models, New Coping Mechanisms

In a rapidly changing environment, work/life balance should also remain a priority, experts agree. While Fleurence has found flexibility within her small company's remote team, IQVIA's global workforce includes many configurations, some of them hybrid. To get a sense of how that's working out, IQVIA runs surveys at least twice a year to uncover and address employee stressors.

"Within our company, every country runs its own wellness program," Parmenter said. "In India, there's a fund for teams to go away and have days out from work. In other parts of the world, the local office might have a summer fair or a holiday party. And teams anywhere might have Friday coffee events."

The same kinds of initiatives, she said, along with perks such as employer-sponsored gym memberships, are being implemented for HEOR workers across the field. And at most companies, Parmenter said, mental-health helplines are part of employment packages.

"In some ways, it's harder now with hybrid working to manage the well-being of a workforce, because we don't see people as often," she said. "However, it's something we need to continue to focus on, because this can be quite a complex world to navigate while balancing all our responsibilities."

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By the Numbers: Empowering HEOR's Workforce

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Doable, Proven, Measurable: 3 Ways to Build HEOR Skills That Work

PROJECT	OUTCOME
1 **Embed fellows inside decision-making teams (70%–100% onsite)** — A Health System Impact-style fellowship places trainees in payer/provider/regulator teams with dual mentorship to deliver training tied to real decisions.	100% employment for 87 alumni; 92% remained in-country and 22% were hired by their host organization.
2 **Run "Ambassador" case-based workshops** — Pairing a clinician with an HTA expert to walk teams through real cases builds skills in quick evidence synthesis, communicating uncertainty, and applying HTA to decisions.	11 sessions; 130 participants; 99% rated useful; 35% reported important practice changes.
3 **Train on machine learning-assisted evidence screening** — Use short, hands-on labs with Rayyan, Abstrackr, and the Cochrane classifier to teach applied triage skills so teams screen faster without sacrificing rigor.	~40% average time saved with Rayyan; ~67% fewer title/abstract screens with Abstrackr.

How Cross-Functional Collaboration Fosters Innovation

Building HEOR Skills—What the ISPOR Competencies Study Says

6 job roles make up 70% of the **HEOR workforce**: HEOR generalist (29%), health economist (18%), HEOR manager (8%), HTA specialist (8%), RWE specialist (7%), PRO/COA specialist (6%).

14/31 The ISPOR **HEOR Competencies** Framework defines 41 skills across **13 domains**.

90% General member survey (n = 493) **found >90% rated** economic modeling, HTA evidence, statistics, and real-world evidence as **critical skills**.

87% The most **job-relevant domain** is communication and influence (87%), highlighting the **importance of soft skills**.

Capturing the True Impact of Duchenne Muscular Dystrophy: How Clinical Evidence and Societal Value Should Evolve for Better Healthcare Decisions

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KEY TAKEAWAYS

Evolving health technology assessment (HTA) frameworks should explicitly incorporate caregiver burden and utility, societal costs, bereavement disutility, and supported use of high-quality natural history data to inform long-term outcomes in rare diseases such as Duchenne muscular dystrophy (DMD).

Broader, preference-based tools are essential to capture what truly matters to patients and families.

The US FDA has accepted matched Natural History Data as confirmatory evidence of clinical findings in DMD, marking a shift toward more pragmatic approaches for assessing long-term outcomes in rare diseases. HTA bodies are beginning to follow, although broader adoption and harmonization are still needed.

Duchenne muscular dystrophy (DMD) is a rare, lethal genetic progressive neuromuscular disease characterized by progressive muscle degeneration, loss of ambulation, and multisystem complications. Beyond the detrimental impact on patients, DMD imposes substantial burden on caregivers, families, and society.

While clinical measures capture disease progression, they often miss the lived experience. This article highlights why health technology assessment (HTA) frameworks must evolve to reflect both measurable outcomes and what matters most to patients and families.

> Slowing down disease progression maintains independence and protects critical developmental periods in childhood. This is important when assessing treatment benefits.

DMD is typically diagnosed in early childhood, with signs of muscle weakness. Patients progressively lose lower limb function, resulting in loss of ambulation (LoA), followed by loss of upper limb function, cardiorespiratory deterioration, and eventually premature death. Measuring this trajectory is fundamental in managing care and evaluating treatment effectiveness.

Validated clinical endpoints are used in both trials and practice to monitor disease status and treatment response. One of the most common endpoints is the 4-stair climb (4SC). A slower 4SC correlates with a reduced participation in physical and social activities of daily living[1] and is predictive of an LoA.[2]

North Star Ambulatory Assessment (NSAA), time to rise (TTR), and 6-minute walk test (6MWT) each reflect different aspects of disease progression. NSAA directly correlates with increased risk of LoA,[3] TTR as an early prognostic factor of disease progression,[4] and 6MWT as a predictor of disease progression.[5]

As well as predicting clinical value, these endpoints carry practical significance. NSAA reflects tasks related to dressing, bathing, and general mobility. The 4SC indicates when a child can no longer safely climb stairs. These clinical tests, while quantitative, reflect patients' daily functioning and quality of life (QoL).

As patients progress through different stages of DMD, the relevance of each functional domain shifts. During early stages, walking ability may dominate concerns. Later, the ability to sit upright or maintain minimal arm function becomes essential for communication, independence, and self-care. Delaying early milestones has been associated with the postponement of later milestones.

When designing successful clinical trials, the primary endpoint, patient population definition, and duration are key factors. DMD trial design must consider how to align endpoints with both the investigational treatment's mechanism of action and the specific patient population. If outcomes are selected without regard for baseline function, age, or disease trajectory, results risk missing a treatment difference when one exists.

Furthermore, following patients' long-term outcomes takes longer than most trials, with evidence gaps necessitating reliance on well-curated, controlled, and high-quality natural history data (NHD).

The United States Food and Drug Administration (FDA) has accepted

matched NHD as confirmatory evidence of clinical findings in DMD,[6] marking a shift toward more pragmatic approaches for assessing long-term outcomes in rare diseases. HTA bodies are beginning to follow, although broader adoption and harmonization are still needed.

Families value stability and preserving function, giving patients and caregivers time to adapt. Delaying the need for hoists or ventilation, preserving the ability to weight-bear, feed oneself, or be able to turn in bed or remain upright can have disproportionate value to patients and their caregivers that traditional quality-adjusted life year (QALY) measures often miss. Failure to consider the patient's lived experience risks undervaluing therapies slowing disease progression.

Slowing down disease progression maintains independence and protects critical developmental periods in childhood. This is important when assessing treatment benefits.

The economic and societal burden of DMD is substantial. Caregivers face income loss (current, future, and pension), home adaptation costs, and considerable emotional, physical, and psychological strain, which increases as the child ages. Caregivers' mental health and QoL are too often overlooked.[7] Family relationships are often impacted (eg, with UK divorce rates at 87% for parents of children with physical disabilities[8]), and siblings grow up in the shadow of the condition. As the disease progresses, care complexity increases for both families and systems.

The cost and complexity of caring for patients increase as the patients' disease progresses, for both the families and society.[7]

The true cost of DMD extends beyond direct healthcare costs, impacting education, social care, and government budget. Yet HTA frameworks rarely incorporate these broader value elements in reimbursement decisions.[9] Recognizing these gaps, methodological evolution of evidence generation is underway in DMD, including quantifying informal care costs, work absenteeism and presenteeism (valued through human-capital approach), and loss of leisure time (eg, informal care costs estimated using proxy good or opportunity costs methods). Some HTA agencies now recommend including these costs; however, more guidance is needed.

Paradoxically, DMD cost-effectiveness models including caregiver health spillovers may show reduced cost-effectiveness for life-extending therapies. Prolonging survival with high care demands increases caregiver disutility, potentially offsetting QALY gains for patients by losses to caregivers. This trade-off of extending life versus increasing caregiver burden is known as the "caregiver QALY trap."[10]

The cost and complexity of caring for patients increase as the patients' disease progresses, for both the families and society.

Several methodological innovations begin to address this: time-dependent bereavement effects, treatment-specific caregiver utilities, and one-off QALY losses associated with major transitions.

Methodologies must evolve to reflect the true burden of DMD and the value of innovation. NHD play a central role. When properly designed and matched for baseline characteristics, NHD offer a powerful alternative to life-long placebo follow-up, especially in rare diseases where such designs may be ethically or practically impossible. In DMD, NHD are increasingly used as comparators to assess long-term outcomes like LoA, loss of upper limb function, or ventilation dependency. These approaches are gaining recognition among regulatory and HTA bodies, but more consistency is needed.

Preference-based measures, such as DMD-QoL-8D and those developed by Project HERCULES, aim to capture dimensions that matter to patients and caregivers: autonomy, social participation, and psychological well-being. These are often missed by generic QoL instruments. Without such measures systematically captured and incorporated into economic models, HTA will continue to undervalue treatments that deliver stability, delay loss, or reduce caregiver stress.

Evolving HTA frameworks should explicitly incorporate caregiver burden and utility, societal costs, bereavement disutility, and supported use of high-quality NHD to inform long-term outcomes in rare diseases such as DMD.

Broader, preference-based tools are essential to capture what truly matters to patients and families.

Ultimately, the value of DMD treatments must be valued based on their real-world impact on both patients and their families. The physical, emotional, and economic toll on caregivers demands formal recognition in HTA cost and QoL dimensions. The growing acceptance of reliable NHD as a comparator reflects a pragmatic evolution to rare diseases' evidence challenges. Properly matched NHD enable evaluation of long-term treatment effects not captured in clinical trials. Embracing both methodological evolution and real-world relevance will inform and empower decision makers to recognize the full impact of DMD and support innovation where it matters most to society.

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Breaking Boundaries: Repositioning HEOR for Broader Impact

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KEY TAKEAWAYS

The evolving healthcare landscape requires health economics and outcomes research (HEOR) teams to broaden their role beyond traditional payer and health technology assessment submissions, collaborating closely with Medical Affairs and Market Access to shape strategy and communication aligned with the realities of today's healthcare system.

HEOR professionals must go beyond scientific expertise, investing in training and skills to effectively communicate evidence in ways that resonate with diverse stakeholders, making their insights more understandable, actionable, and impactful across the healthcare ecosystem.

Measurable outcomes are critical for HEOR; consistently tracking and showcasing evidence-driven wins directly demonstrates the value and relevance of their work to the organization.

As many pharmaceutical organizations have restructured, some health economic and outcomes (HEOR) teams have been integrated with reporting lines into Medical Affairs or Market Access. In some cases, the accountability of ensuring timely delivery of integrated evidence has shifted to Medical Affairs, while the responsibility for evidence generation resides with HEOR or real-world evidence (RWE) teams.

This organizational restructuring raises questions that cannot be ignored. *"Is HEOR losing clout in pharma? Is the role of HEOR evolving? Is the role of HEOR diluted if it is not a stand-alone group?" What are the real issues that HEOR should focus on?*

This article explores the evolving role of HEOR in an era where the traditional role centered on payers and health technology assessment (HTA) submissions is no longer sufficient.

Redefining Value of HEOR

For decades, HEOR built its reputation on being a rigorous analytical function, designing economic models and generating outcomes data to support payer decision making. With this laser focus on access and reimbursement, by those of us who have seen HEOR evolve, we have amplified the "HE" (health economics) in HEOR at the expense of the "OR" (outcomes research). It is time to broaden our aperture.

The realities of today's healthcare ecosystem require a much broader view, one that embraces care delivery, system-level efficiencies, and long-term health outcomes. Payers and providers are much more integrated today, and key opinion leaders and medical societies have a greater influence on payer and access decisions.

Real-World Evidence Is Not Just for HTA

Physicians increasingly seek evidence that reflects the complexities of real-life care, including comorbidities, adherence patterns, and care disparities. HEOR teams that focus exclusively on payer-focused deliverables risk missing the opportunity to shape clinical behavior.

Furthermore, as the reach of digital health tools and remote monitoring expands, the volume and granularity of real-world data have exploded. HEOR should play a central role in analyzing these data to inform guideline development, facilitate comparative effectiveness, and influence quality improvement initiatives. However, this will happen only if we recognize the needs of these new audiences as part of our remit.

The Problem Is Not Structure, It Is Strategy

The integration of HEOR into Medical Affairs/Market Access is an opportunity to become more impactful. If HEOR professionals accept the invitation to contribute as strategic architects by shaping evidence generation, aligning timelines with market forces, and framing value stories, then the relevance of HEOR expands.

> The integration of HEOR into Medical Affairs/Market Access is an opportunity to become more impactful.

Our focus should be to link evidence generation and evidence communication. HEOR should leverage this alignment to influence medical strategy and shape value narratives that resonate with all stakeholders. This shift is not disempowering, it is empowering, when seized proactively. However, if we simply execute requests, our influence will continue to diminish.

In organizations where this model has been adopted, HEOR professionals sit alongside medical teams, country affiliates, and market access leads. They provide strategic insights and develop tools that shape how value is communicated at congresses, advisory

boards, in peer-reviewed publications, and one-on-one engagements with access decision makers. In addition, the partnership between Market Access, Medical Affairs, and HEOR continues to grow when a collaborative, evidence-driven approach to value demonstration and HTA submissions becomes the accepted approach.

> The real question is not whether HEOR should evolve; it is whether we will take control of its evolution.

Capturing "evidence wins" should be part of routine practice. Did a particular analysis shape pricing negotiations? Was a real-world study referenced in a payer meeting or advisory board? These moments demonstrate the application-focused value of HEOR and should be tracked, socialized, and celebrated within the company.

Is the Impact of HEOR Weakening?
We identified 3 factors listed below.

Communication Gaps: Highly technical HEOR deliverables are often complex and difficult to interpret, hence misunderstood or underutilized by nonspecialist internal stakeholders. This may have resulted in data that was "lost in translation," where the potential impact of the evidence was muted or, in some cases, nullified.

Timing Misalignment and Strategic Disconnect: HEOR evidence-generation studies, if planned independently, may not be aligned with the product's value or generated too late, leading to evidence that was "not relevant," did not support the storyline of the product, or simply delivered too late. Without deliberate integration, HEOR evidence generation risks being deprioritized based on misaligned research interests, rather than evidence gaps based on market needs.

Failure to Pivot: Endpoint requirements have evolved with changing landscapes. Providers, payers, and regulators have been requesting patient-centered outcomes that are meaningful from a clinical practice perspective. Surrogate makers and novel endpoints have advanced science and drug development; however, have they been linked to endpoints used in clinical practice? This requires Medical Affairs, Market Access, and HEOR functions to collaborate with external stakeholders to scope and execute relevant evidence-generation studies.

The Path Forward: 4 Imperatives
Below are the 4 imperatives for HEOR in our future journey.

Intentional Strategic Collaboration

- *Partner Early, Partner Deeply:* Embed product champions (including HEOR, Market Access, and Medical Affairs) into value strategy teams from the earliest stages of asset planning.
- *Intentional Engagement:* Actively contribute as an asset champion by participating in key meetings, including brand reviews, asset value teams, study design, launch and HTA readiness, and field team planning for access and reimbursement.
- *Knowledge Exchange:* Hold "teach-ins" and workshops for internal stakeholders in collaboration with other relevant functions.
- *Expand the Definition of Stakeholder:* Go beyond payers; engage prescribers, patients, and society at large.

Master Translation of Evidence

- *Training:* Upskill by developing an expertise in visualization tools, storytelling skills, and audience-specific translation materials.
- *Deliverable Redesign:* Create modular value communication tools tailored to specific stakeholders—such as customized slide decks, infographics, objection handlers, and frequently asked questions documents.
- *Peer Learning and Coaching:* Establish a program to support evidence generation leaders (HEOR, Medical Affairs, etc) in building skills for developing compelling, value-focused narratives.
- *Evidence Feedback Loop:* Work closely with customer-facing teams to deliver evidence-based resources to external stakeholders; share insights with internal evidence-generation teams.

Cross-functional Strategic Planning

- *Integrated Approach to Evidence Planning:* Take an integrated approach to evidence generation to create a unified, efficient, and high-value strategy for the product(s). Harmonize across functions and geographies beyond the regulatory trials for the asset/product.
- *Evidence Gap Assessment:* Execute annual workshops for "evidence gap assessments."
- *Rapid Response Teams:* Deploy cross-functional squads for high-priority deliverables, providing concurrent access to necessary tools, endpoints, and analytical support.

Performance Metrics and Success Stories

- *Measurable Impact:* Track HTA wins, guideline uptake, field tool use, etc, and proactively communicate success stories.
- *Case Illustrations:* Profile success stories where an integrated approach to delivery directly supported favorable access or price negotiations.

Recommendations: Concrete Next Steps
For organizational leaders:

- Redefine HEOR roles to include strategy, communication, and stakeholder engagement.
- Invest in cross-functional integration platforms, communication tools, and planning forums that facilitate collaboration.
- Create a collaborative work environment with clear accountability.
- Build key performance indicators for evidence translation and uptake.
- Allow time for upskilling and "field immersion" (eg, HEOR shadowing field medical and sales representatives, Medical Affairs country affiliates, and other functions as relevant).
- Cocreate an "integration checklist" with other functions: clarifying decision points, deliverable formats, and evidence needs.
- Celebrate success and share best practices along with key learnings.

For HEOR professionals:

- Volunteer for early engagement.
- Communicate with precision and passion.
- Ask how your work will be used and how it will make an impact, not just how it will be designed.
- Practice communicating your findings to non-HEOR specialists.
- Have a culture of continuous learning by incorporating insights and feedback.
- Track downstream use of your deliverables—and share the wins internally and externally.

Conclusion

The real question is not whether HEOR should evolve; it is whether we will take control of its evolution. Will it be a passive function waiting to be activated, or an active architect guiding evidence strategy across the development life cycle?

As highlighted in ISPOR's Amplify HEOR initiative, our profession is at a crossroads: we can either double down on our insular technical excellence or step up as the translator of value across diverse stakeholder groups. As Jens Grueger notes, "We need to focus more on that communication piece, not only from our leaders in HEOR, but also begin training our young colleagues to recognize that communication is an important piece for what they're doing."

Similarly, Darius Lakdawalla's research on Medicare obesity coverage underscores the need to make value concrete. Rather than defending costs, we must link economic evidence to human impact, longevity, quality of life, and access. In doing so, HEOR can ensure its future by proving its present-day relevance.

Call to Action: As the landscape continues to evolve, HEOR can demonstrate not only its relevance but its necessity by embracing the imperatives outlined above, which include broader roles, refining communication, engaging cross-functional teams, and measuring impact. Let's work together to reposition HEOR for broader impact!

The Comprehensive Assessment of Technologies for Child Health (CATCH) Framework: Deliberating on the Whole Child

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KEY TAKEAWAYS

Health technology assessments (HTA) for child health technologies are currently constrained by evidentiary and methodological challenges.

Early, robust, and systematic deliberation in HTA processes to capture highly diverse societal perspectives on the unique needs of children may promote wide-lens, societally responsive funding decisions.

The Comprehensive Assessment of Technologies for Child Health Framework is a child-tailored, deliberation-driven, multicriteria decision-analysis–based value assessment framework. It is intended to bolster rather than replace established HTA processes, such as expert committees and cost-effectiveness analysis.

Introduction

It has long been recognized that conventional methods of health technology assessment (HTA) do not serve children and youth well, resulting in missed opportunities for health outcome improvements and diminished access to therapeutics compared to adults.[1,2] Why is making HTA decisions for children more difficult and more complex than for adults? The challenges to determining the value of novel drugs for children can be broadly categorized as: 1) evidentiary limitations, particularly the paucity of randomized clinical trial data, related to difficulties in trial enrollment and the unique biology and epidemiology of childhood diseases; 2) methodological constraints of standard economic evaluation, given that techniques based on adult- or population-level beneficiaries do not estimate child-related utilities and externalities well or at all; and 3) ethical shortfalls, such as exclusionary procedures (eg, using proxies' values rather than children's) and persistent inequities (eg, those caused by deleterious consequences of treatments on physical and cognitive development).

However, the field of HTA is evolving in exciting ways to define value better in the new paradigms of medical research and product development.[3] Child-related utility and outcomes measures are expanding.[4] Consultations with patients and caregivers are becoming increasingly integrated into evaluations to complement the expert views of clinicians and economists.[5] Moreover, to promote transparent and formalized (rather than intuitive) methods in complex healthcare decision making, multicriteria decision-analysis (MCDA) techniques are being considered to supplement traditional but more narrowly focused quantitative methods, such as cost-effectiveness analysis.[6]

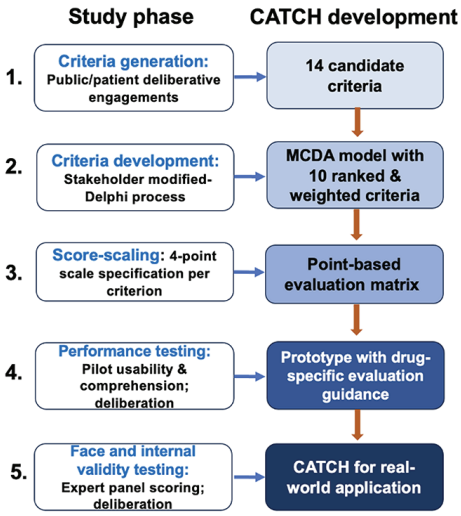
In this article, we present key insights into the deliberative aspects of our research, which generated a wide range of perspectives to imbue an additive MCDA model with societal values specifically related to the health and well-being of children. The Comprehensive Assessment of Technologies for Child Health (CATCH) Framework is a child-tailored value assessment framework based on that model, operationalizing criteria deemed important to the holistic flourishing of children, beyond the immediate impacts of treatment. We apply it in the case of childhood cancer precision therapies.

This work was previously presented in parts at ISPOR Europe 2023 and in 2 peer-reviewed articles, including in the July 2024 issue of *Value in Health*.[7-9]

A multistage process propelled by deliberative engagement

CATCH was developed through multiple iterative phases, initiated by early deliberations with the general public in "Citizen Panels," a model of deliberative public engagement, to gather views for identifying assessment criteria. Criteria and the MCDA model were refined by a modified Delphi process and validated through 2 rounds of applied testing and deliberation (**Figure 1**).

Figure 1. Development of the Comprehensive Assessment of Technologies for Child Health (CATCH) Framework



Citizen views on children's importance in society and the HTA process

The 3 Citizen Panels were broadly representative of the Canadian public but included 1 panel purposively composed of young people 16-22 years of age to gain their specific perspectives. To enable informed and meaningful deliberation, we provided technical information briefs, overviews of the Canadian drug regulatory and funding systems, and personal testimony from a caregiver with lived experience of childhood cancer. Participants thought "Childhood Distinctions," "Voice," "One versus Many," and "Health System Governance" should characterize the tableau of child-tailored HTA. Within these themes, 14 candidate criteria for HTA were delineated (**Figure 2**).

Given the transformative potential of precision drugs, it is perhaps unsurprising that Hope emerged as a pervasive subtheme (criterion), spanning multiple themes. *Fair Innings*, a concept stemming from health economics, was largely accepted as a valid basis of assessment, expressed by 1 participant thus, "...much as you love your grandmother, they've already lived up to their potential...rather than someone who is 10 years old."

Shaping citizen values for MCDA by HTA stakeholders

Stakeholders including caregivers, health economists, bioethicists, and representatives from industry and government regulatory and reimbursement agencies were next engaged in a modified-Delphi process. Over 2 surveys and consensus-building deliberations, the broad-based and somewhat amorphous citizen values were honed and given finer-grained definitions to create criteria suitable for a MCDA model. Criteria provoking controversy were explored through group argumentation. Contrary to general-public sentiments, these participants rejected the inclusion of *Hope*, citing the difficulty of articulating it for HTA contexts. *Fair Innings* received weak endorsement due to ethical objections to the potential prioritization of younger over older populations; participants nevertheless agreed on its inclusion.

> The CATCH Framework is a child-tailored value assessment framework based on that model, operationalizing criteria deemed important to the holistic flourishing of children.

The resulting 10 criteria were considered to be nonoverlapping, important, and feasible to implement, comprising criteria that were common to HTA (*Effectiveness, Therapeutic Safety, Disease Severity, Unmet Need*), less common (*Rarity, Equity*), and, notably, new and specific to children and youth (*Child-Related Health Quality-of-Life, Family Impacts, Childhood Development, Fair Innings*). We allocated a budget of weights based on twice-solicited rank-order importance and quantification of "best-worst" and "discard-keep" opinions.

The wide multidisciplinarity of stakeholders, building on a foundation of public values, provided an enlarged lens uncommon to HTA processes that was nevertheless bounded by informed opinion and experience in weighing benefits and costs of health interventions. The CATCH Framework thus far described may therefore serve as a model for the role of deliberative engagement in early stage appraisal processes.

Putting the CATCH Framework to the test

In finishing the CATCH Framework for application to appraisal, we developed a 4-point score scale for each criterion. A weighted-sum score (weight times score aggregated over 10 criteria) indicated the priority level for funding the appraised drug. Three clinicians expert in pediatric oncology drugs piloted CATCH for 10 precision drugs, by which we were able to further refine and clarify the criteria (including names) and to adjust scoring metrics and levels. In 2 deliberative rounds, *Fair Innings* became the more intuitively grasped *Fair Share of Life*, and *Childhood Development* became *Life Course Development*, better capturing unique child-specific concerns with therapies having late-effect impacts (eg, reduced fertility, secondary neoplasms). The clinicians suggested alternative scoring subcategories to capture qualitative evidence for *Child-Specific Health-Related Quality-of-Life*, given a typical lack of quantitative evidence, and to distinguish between additive and substitutive therapies in *Therapeutic Safety*. **Figure 3** shows the final set

Figure 2. CATCH Phase I themes and criteria

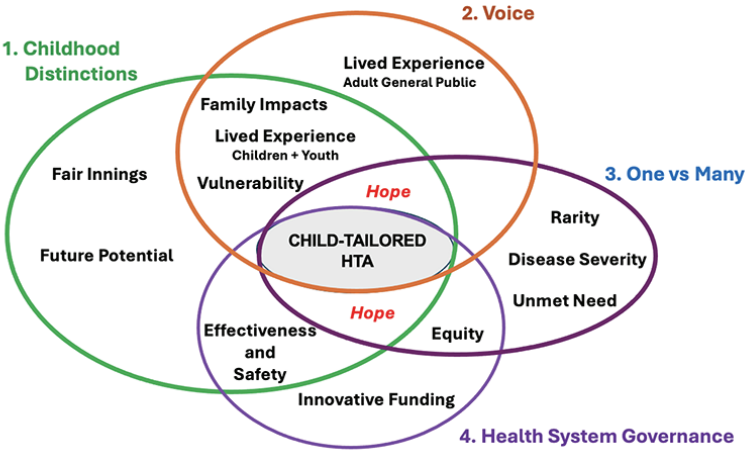
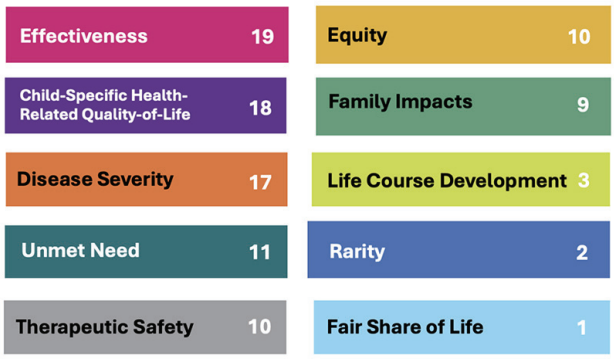


Figure 3. Final CATCH criteria and weights



of criteria and their respective weights.

The refined CATCH Framework, **Table 1**, was then validated by a group of pediatric oncologists and a pharmacist in a mock HTA appraisal committee. Participants were provided with CATCH and clinical evidence briefs for blinatumomab (indicated for relapsed B-cell acute lymphoblastic leukemia) and brentuximab vedotin (high-risk Hodgkin's lymphoma), accompanied by guidance on relating the individual evidence components to specific criteria and scoring levels. Participants scored individually, then the group was convened to share their rationales and to arrive at consensus scores and, consequently, funding recommendations. The group affirmed that in their expert opinions, the novel, child-specific criteria were relevant and meaningful for assessing the 2 targeted therapies on important health and well-being outcomes. Furthermore, they affirmed the coherence of the standard and child-specific criteria together within the CATCH Framework.

> We found that members of the public and clinical and technical experts were eager to ponder and discuss the whole child and how to decide achieving the best outcomes for them.

We completed our study by varying the weights of the child-specific criteria to examine the impact on the weighted-sum scores. As there were no resulting significant shifts in funding recommendations, we concluded that CATCH was robust for application to childhood cancers.

Table 1. The Comprehensive Assessment of Technologies for Child Health (CATCH) Framework

			SCORE			
CRITERIA	**DEFINITION**	**WEIGHT**	**0 (No improvement)**	**1 (Minimal improvement)**	**2 (Moderate improvement)**	**3 (Significant improvement)**
Effectiveness	The ability of a treatment to improve key disease-specific outcomes, compared to existing treatments or standards of care.	19	No evidence of improvement (or evidence of worsening) in relevant disease-specific outcomes	Any evidence of improved disease stability/delayed disease progression compared with standard treatments	Clinically and/or statistically significant improvement in disease control (eg, response rate, progression-free survival, key measures of disease activity), or short-term (<1 year) survival	Clinically and/or statistically significant improvement in 2-year survival, or curative potential with evidence of 1-year survival benefit
Child-Specific Health-Related Quality of Life (QoL)	The impact of a new treatment on a child's well-being, including social, emotional, and physical dimensions of daily life, in regard to a measured minimally important difference (MID) or as qualitatively reported by patients/caregivers, compared to existing treatments or standards of care.	18	***Where quantitative evidence exists***			
			Decrements in QoL during therapy	Maintained or improved QoL scores at or above MID during or after therapy in <1/3 of patients	Maintained or improved QoL scores at or above MID during or after therapy in >1/3 of patients	Maintained or improved QoL scores at or above MID during or after therapy in >1/2 of patients
			In absence of quantitative evidence, but qualitative evidence exists			
			Decrements in QoL during therapy	Qualitative evidence of improved or maintained QoL during or after therapy in minority of patients, without notable decrements in QoL	Qualitative evidence of maintained QoL during or after therapy in most patients	Qualitative evidence of improvements in QoL during or after therapy in most patients
Disease Severity	The presence or extensiveness of a disease in the body, as measured by its mortality rate and/or degree of morbidity.	17	No disease-related mortality and minimal impact of disease-related morbidity on daily function	Childhood disease-related mortality with current treatments <25%, or morbidity limiting daily function in <25% of patients	Childhood disease-related mortality with current treatments >25% and <50%, or morbidity limiting daily function in >25% and <50% of patients	Childhood disease-related mortality with current treatments >50%, or morbidity limiting daily function in >50% of patients
Unmet Need	The availability or accessibility of appropriate therapeutic alternatives for children and youth with the relevant disease/treatment indication.	11	Available therapies adequately meet patient needs	Alternative effective therapies exist, but ongoing need for improvement in disease-specific outcomes	Few existing appropriate therapies for indication, with ongoing need for improvement in disease-specific outcomes	No existing appropriate therapies for indication
Therapeutic Safety	The absence of acute and/or long-term safety concerns or adverse effects (AEs) of a treatment, compared to existing treatments or standards of care.	10	***Additive therapies***			
			Increased burden of Grade 3-4 treatment-related AEs compared to existing standards of care	Increased burden of Grade 1-2 treatment-related AEs compared to existing standards of care	No increased burden of Grade 3-4 treatment-related AEs compared to existing standards of care	No increased burden of treatment-related AEs compared to existing standards of care
			Substitutive therapies			
			Increased burden of treatment-related AEs compared to existing standards of care	No increased burden of treatment-related AEs compared to existing standards of care	Reduction in Grade 1-2 AEs compared to existing standards of care, no increase in Grade 3-4 AEs	Reduction in Grade 3-4 treatment-related AEs compared to existing standards of care
Equity	The impact of the new treatment on the health of vulnerable or marginalized populations, particularly those who are unable to voice their preferences or needs, compared to existing treatments or standards of care.	10	Treatment not indicated for disease affecting marginalized or vulnerable population, or expected benefits not anticipated to resolve/address underlying causes of outcome disparities among populations	Treatment indicated for disease affecting marginalized or vulnerable population, expected benefits to accrue to disadvantaged population in similar proportion as to other populations with the disease	Treatment indicated for disease affecting marginalized or vulnerable population, expected benefits to accrue more to disadvantaged population (eg, oral treatment that enables better access geographically)	Treatment indicated for disease uniquely or disproportionately affecting marginalized or vulnerable population, expected benefits to accrue primarily to disadvantaged population
Family Impacts	The impact of the new treatment on the health and well-being of the patient's family, including their parents or primary caregivers and siblings, compared to existing treatments or standards of care.	9	No improvement in family health and well-being as compared to standard treatment	Treatment expected to ease logistics of care but without clear benefits to outcomes (eg, better quality of life, mental health status, economic/educational productivity, and/or social cohesion among patient family members)	Treatment expected to ease logistics of care with anticipated benefits in 1 or more outcomes (eg, better quality of life, mental health status, economic/educational productivity, and/or social cohesion among patient family members)	Treatment expected to enable better quality of life, mental health status, economic/educational productivity, and/or social cohesion among patient family members
Life-course Development	The impact of the treatment on the growth and maturation of perceptual, intellectual, emotional, behavioral, and physical capabilities and functioning that occur from infancy through adulthood, compared to existing treatments or standards of care.	3	Greater burden of anticipated or proven risks to life-course health	No anticipated or proven reduction in deleterious life-course health impacts of disease or current therapies, but also no anticipated or proven increased risk to life-course health as compared with current standards of care	Treatment anticipated or proven to reduce mild-to-moderate deleterious life-course health impacts of disease or current therapies in 1 dimension (perceptual, intellectual, emotional, behavioral, physical)	Treatment anticipated or proven to reduce any moderate-to-severe deleterious life-course health impacts of disease or current therapies, or reduce mild-to-moderate impacts in >1 dimension (perceptual, intellectual, emotional, behavioral, physical)
Rarity	A disease or medical condition with a prevalence of <1 per 2000 persons.	2	Treatment targets a disease with high prevalence	Treatment targets a disease with moderate prevalence	Treatment targets a rare disease/condition (<1/2000)	Treatment targets an ultrarare disease/condition (<1/50,000)
Fair Share of Life	The prioritization of treatment for those who have experienced, or are expected to experience, comparatively less of a normal span of healthy life, in hopes of giving everyone an equal chance at a "fair share" in life. The implication of this is that saving 1 year of life for a younger person is valued more than saving 1 year of life at comparable health for an older person.	1	No anticipated or proven benefits to populations who have experienced comparatively less of a healthy span of expected life	Treatment is anticipated or proven to benefit populations who have experienced comparatively less of a healthy span of expected life (irrespective of equal or greater benefits to populations who have had a greater share of expected life)	Treatment is anticipated or proven to primarily benefit populations who have experienced comparatively less of a healthy span of expected life	Treatment is anticipated or proven to primarily benefit populations who have experienced comparatively less of a healthy span of expected life
		100				

The intrinsic benefit of CATCH for application to other childhood conditions is, we believe, the early and explicit focus on values for child health and well-being, sustained through iterative, progressive deliberation with as wide a stakeholder perspective as possible.

Implications for HTA organizations

In the current environment of HTA evolution, there is an opportunity to consider evaluation methods that not only embrace wider notions of value in child health technologies but also increase decisional legitimacy. We found that members of the public and clinical and technical experts were eager to ponder and discuss the whole child and how to decide achieving the best outcomes for them. For example, participants expressed (clinical) Effectiveness as the premier criterion (weight = 19, out of 100 points), thus affirming the legitimacy of HTA agencies standardly using this criterion as a "first hurdle." Importantly, *Child-Specific Health-Related Quality of Life* ranked second (weight = 18), over another standard criterion, Therapeutic Safety, indicating participants' high regard for the child's entire treatment experience.

In the CATCH Framework, we have created a structure and heuristic to strengthen and develop deliberative engagement in HTA assessment of child health technologies. It delineates a role for stakeholder participants, including those who do not have an immediate stake in HTA decisions, to provide societal values and to identify assessment criteria for which evidence must be gathered and debated during decision making, furthering the international development of evidence-informed HTA deliberation.[10]

The challenge remains to create a more generalized version of CATCH that could be applied to other childhood conditions. We are currently working toward this goal. Ultimately, we aim to fill a gap in evaluating child health technologies by incorporating a child-tailored value assessment framework based on CATCH in HTA processes in Canada and internationally.

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Are Digital Therapeutics Reshaping the Health Technology Assessment Framework for Health Technologies?

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KEY TAKEAWAYS

While patient insights are rarely determinants in the health technology assessment (HTA) decision making for drugs, there has been a growing awareness of the impact of the patient experience on the treatment outcomes with the increasing presence of digital health technologies (DHTs).

With the emergence of DHTs, patients are increasingly providing insights on their experience with the use of the technologies, which is being linked to the observed clinical outcomes and is driving HTA decision making for DHT funding.

Collaboration among all providers and recipients of care, in addition to the industry and policy makers, is important to foster efforts to advance patient participation in the HTA decision-making process.

The role of patient insights in HTA decision making has historically been limited

Health technology assessment (HTA) is a comprehensive process that evaluates the clinical benefits and economic implications of a health technology through evidence-based research and quantitative analysis. When assessing health technologies, the evaluation of these benefits has traditionally considered the disease background, unmet need, and the clinical and economic value of the product. In situations where the value is weighted against costs, economic evaluations are used to improve the rational and efficient use of financial resources, with the coverage of technologies remaining subject to their cost-effectiveness and affordability in many markets.[1]

In the last few years, the incorporation of the humanistic value of health technologies into the HTA discussion has increased. Elements related to the patient's value are captured through patient-reported outcomes (PROs) in clinical trials, surveys, or studies involving patient inputs. However, these elements tend to impact decision making only when there is high uncertainty or incomplete evidence, or when PRO data are required to build economic models. Therefore, several important patient benefits (such as patients' preference, convenience, symptom experience, treatment satisfaction, etc) may be overlooked in the current HTA frameworks.

The Food and Drug Administration (FDA) has defined digital health technologies (DHTs) as "systems that use computing platforms, connectivity, software, and/or sensors for healthcare and related uses."[2] Examples involve mobile applications, such as CANKADO, which provides patient support, patient diaries, and documentation on PROs for patients suffering from HR+ HER2- metastatic breast cancer. The emergence of DHTs has raised a need to conduct HTAs on these technologies. Several HTA agencies have changed or adjusted their traditional assessment frameworks to adapt to the new specificities of DHTs. Examples include the Gemeinsamer Bundesausschuss (G-BA) in Germany, the National Institute for Health and Care Excellence (NICE) in the United Kingdom (UK), and the Haute Autorité de Santé (HAS) in France.[3] Given the critical link between the health outcomes of DHTs and patients' ability to use the DHTs, there has been a growing awareness of patients' experience with the use of DHTs and of the impact that this has on the safety and efficacy of the DHTs. Consequently, the HTA frameworks of several HTA agencies have been adjusted to further increase the degree at which patient experience is incorporated into the HTA decision-making process.

> "We explore how the increase in the "voice of the patient" in digital health technology assessment is reflected in the evaluations and how it may shape future HTA processes for drugs."

Understanding the ongoing shifting paradigm of the use of patient insights is critical for identifying current trends that may shape the future of HTA decision making

The recent emergence of DHTs and the need to establish an HTA framework in this area have led HTA decision makers to pay more attention to PROs. In this article, we explored how the increase in "voice of the patient" in DHT assessment is reflected in the evaluations and how it may shape future HTA processes for drugs. To identify innovative drugs in Europe, we searched the European Medicines Agency (EMA) database for drugs approved via an accelerated pathway between 2018-2022. Drugs

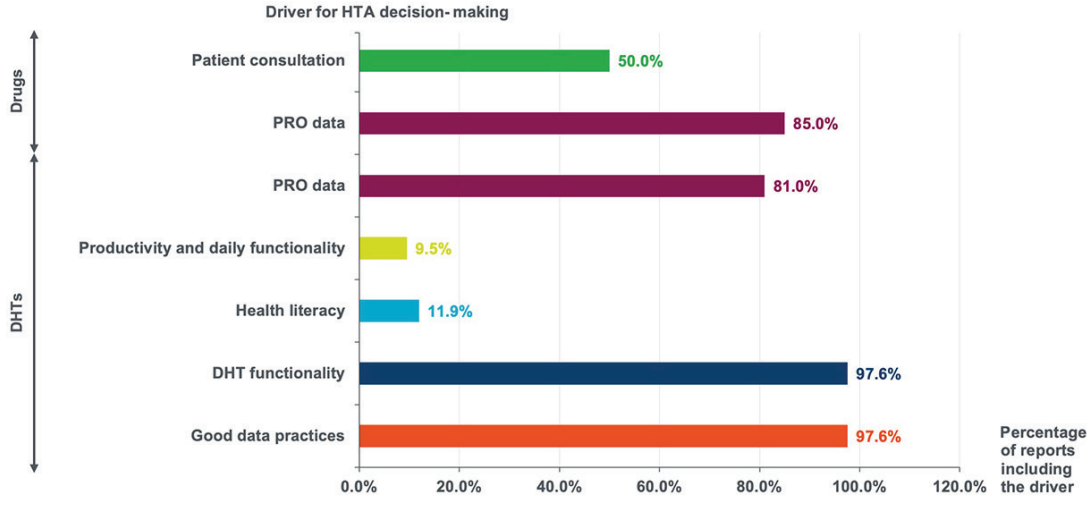
Table 1. Drugs and digital health technologies identified and analyzed

	Active ingredient/App	Target disease	HTA report
Drugs	Odevixibat	Intrahepatic cholestasis	NICE, HAS, G-BA
	Risdiplam	Spinal muscular atrophy	NICE, HAS, G-BA
	Emicizumab	Hemophilia A	NICE, HAS, G-BA
	Budesonide	Esophageal diseases	NICE, HAS
	Patisiran	Familial amyloidosis	NICE, G-BA
	Inotersen	Amyloidosis	NICE, G-BA
	Lanadelumab	Hereditary angioedema	NICE, G-BA
	Ciclosporin	Conjunctivitis, keratitis	HAS
	Betibeglogene autotemcel	Beta-thalassemia	G-BA
DHTs	Before vida	Alcohol mental and behavioral disorders	G-BA
	CANKADO PRO-react onco	Breast cancer	G-BA
	Cara care	Irritable bowel syndrome	G-BA
	Companion patella	Anterior knee pain	G-BA
	Deprexis	Depression	HAS
	Diabeo	Diabetes	HAS
	Edupression	Depression	G-BA
	Elevida	Multiple sclerosis	G-BA
	Elona	Depression	G-BA
	Endo app	Endometriosis	G-BA
	HelloBetter	Diabetes, agoraphobia, panic disorder, pain, sleep disorder, stress and burnout, vaginismus	G-BA
	Invirto	Agoraphobia, social phobias, panic disorder	G-BA
	Kaia COPD	Chronic obstructive pulmonary disease	G-BA

	Active ingredient/App	Target disease	HTA report
DHTs	Kalmeda	Tinnitus aurium	G-BA
	Kranus edera	Impotence of organic origin	G-BA
	Mawendo	Diseases of the patella	G-BA
	Mindable	Panic disorders and agoraphobia	G-BA
	Moovcare poumon	Lung cancer	HAS
	My tinnitus	Tinnitus aurium	G-BA
	Neolexon aphasia	Dysphasia and aphasia, apraxia	G-BA
	Non-smoking hero	Mental and behavioral disorders from tobacco	G-BA
	Novego	Depression	G-BA
	Optimune	Malignant neoplasm of the mammary gland	G-BA
	Oviva direct	Obesity	G-BA
	Pink! Coach	Malignant neoplasm of the mammary gland	G-BA
	Reflex	Gonarthrosis	G-BA
	Selfapy	Depressive and anxiety disorder	G-BA
	sinCephalea	Migraine	G-BA
	Sleepio	Insomnia	NICE
	Somnio	Insomnia	G-BA
	Velibra	Agoraphobia, social phobias, panic disorders, generalized anxiety disorders	G-BA
	Vitadio	Type 2 diabetes	G-BA
	ViViRa	Osteochondrosis	G-BA
	Zanadio	Obesity	G-BA

DHT: Digital health technology; G-BA: Gemeinsamer Bundesausschuss; HAS: Haute Autorité de Santé; HTA: Health technology assessment; NICE: National Institute for Health and Care Excellence

Figure 1. Drivers of health technology assessment decision making identified through health technology assessment appraisals



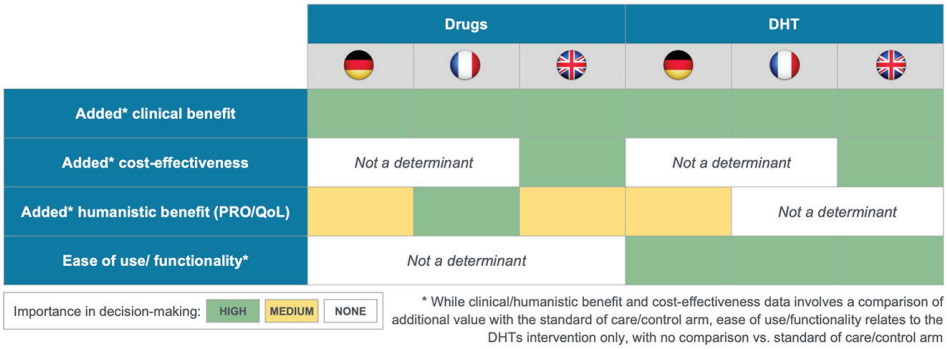
DHT, digital health technology; HTA, health technology assessment; PRO, patient-reported outcomes.

approved via the accelerated pathway by EMA capture drugs with the greatest need for access. Nine drugs in various therapeutic indications were identified. The HTA reports for these drugs were retrieved from the G-BA, NICE, and HAS websites (**Table 1**). We also identified DHTs assessed by these agencies up to 2022, noting that DHTs are approved by each country's regulatory agency, unlike the centralized process for drugs. (**Table 1**). For both innovative drugs and DHTs, we analyzed HTA reports, focusing on patient-related insights and PRO data, including quality of life (QoL) information. Using thematic analysis, we assessed which patient insights were considered in these reports and how often. Finally, we examined the rationale behind HTA decisions to identify patient-related factors that were important determinants for the HTA decisions.

The impact of patient insights on HTA decision making for drugs remains low, with PRO data being increasingly used but having limited influence on the final HTA decision

Our analysis of HTA decisions for both drugs and DHTs reveals interesting trends in how patient perspectives are considered across different technology types. Among the 20 HTA reports identified with published decisions for drugs, 50% and 85% included patient consultation and PRO data, respectively (**Figure 1**). While the inclusion of PRO data in the evaluation was high (85%), it was also evenly distributed across the 3 HTA agencies; in contrast, patient consultations were conducted in 50% of the evaluations by NICE and HAS only. When analyzing the impact of patient consultation and PRO data on the final HTA decision,

Figure 2. Comparisons of the influence of different drivers in health technology assessment decision making



DHT, digital health technology; HTA, health technology assessment; PRO, patient-reported outcomes.

HAS appeared to give significant weight to those factors, while NICE and G-BA seemed to place somewhat less emphasis on them (**Figure 2**). As expected, clinical benefit remained the primary factor in the HTA decisions made, with cost-effectiveness also being an influencing factor, particularly in the United Kingdom.

In contrast with the HTA framework for drugs, patient insights are a main driver for HTA decision making when evaluating DHTs

When it comes to DHTs, we observed a substantial shift in approach by HTA agencies compared to the evaluation of drugs. Patient insights emerged as a crucial driver in HTA decision making for these technologies. The majority of DHT assessments included PRO data (81%) (**Figure 1**). Moreover, new patient-relevant elements were included, specifically the assessment of increases in patients' productivity and daily functioning as well as their ability to understand and use the technology effectively (health literacy). While not directly related to patient experiences, DHT functionality and good data practices were almost universally considered in assessments. These factors indirectly reflect patients' ability and willingness to use the technologies, further emphasizing the patient-centric approach in DHT evaluations. Overall, 98% of analyzed HTAs for DHTs included elements that either directly and/or indirectly considered patient-relevant drivers for decision making (**Figure 1**). Germany's G-BA showed the most diverse range of considerations in their DHT assessments, likely due to the country's pioneering efforts in advancing access to these technologies. NICE and HAS consistently emphasized DHT functionality and data practices in their evaluations.

Overall, similar to the evaluation of drugs, clinical benefit remains the primary factor in the HTA decision making for DHTs. However, a notable distinction emerges in the evaluation of DHTs: ease of use and functionality are consistently strong determinants in decision making across all 3 countries studied. This contrasts with drug assessments, where these factors play no role at all (**Figure 2**). This difference may highlight a new trend: the evaluation of DHTs is evolving toward a more patient-centric approach, diverging from the traditional assessment methods used for pharmaceuticals.

The way forward—placing the patient at the heart of the HTA decision making

While patient insights remain as an optional component of clinical trials, health stakeholders are advocating for the inclusion of these insights, going beyond the traditional QoL metrics commonly included. This aims to elevate the role of patients in the research and development (R&D) process of health technologies, with subsequent implications on the decision making for their marketing authorization and financing. As the R&D of DHTs increases, with subsequent increases in the number of DHTs launched and requesting funding, the number of clinical trials that place patients' ability to use and understand health technologies and the importance of adherence and compliance in direct relation to clinical outcomes and the value of health technologies increases.

The ongoing increase in HTA appraisals for DHTs will likely boost the number of evidence packages that focus not only on traditional disease, clinical, and economic value arguments but also on patient insights and their understanding of the technology and the value of compliance/adherence. Furthermore, patients' transition from being passive recipients of care to valuable sources of data to support the value of products has the potential of empowering patients to become more active in their participation in decision-making processes that involve their own health. Patient-centered evidence packages, in combination with patient empowerment, may drive advocacy efforts to increase the role that patient insights play across all levels of access to health technologies. This includes patient participation at the HTA decision making level through participation in HTA consultation processes. This will likely be further supported by existing processes in some countries, such as Canada and the UK, where patient consultation is already part of the HTA decision-making process.

> When it comes to DHTs, we observed a substantial shift in approach by HTA agencies compared to the evaluation of drugs. Patient insights emerged as a crucial driver in HTA decision making for these technologies.

Nonetheless, quantifying patient insights through robust metrics can be challenging, particularly in vulnerable populations (eg, children, disabled people). Furthermore, the incorporation and/or amendment of new and/or existing guidelines, policies, and/or regulations defining the frameworks for HTA decision-making processes can be time consuming, with governments lacking the resources required. Methodological variability across and

within countries may further increase the challenges for the adoption of patient insights within countries' HTA frameworks.

Collaboration among all providers, recipients of care, industry, and policy makers is important to foster efforts to advance patient insights in the HTA decision-making process. Governments should perceive the allocation of resources to support these efforts as an investment that can maximize the efficiency of the HTA decision-making process. Moreover, the industry should consider switching traditional R&D processes to incorporate patient insights earlier in the clinical development program.

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Fast-Tracking Digital Health: How Accelerated Coverage Pathways for Innovation Are Paving the Way for Innovation

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KEY TAKEAWAYS

The integration of digital health technologies into healthcare systems hinges on accelerated coverage pathways for innovation (ACPIs), which bridge gaps in reimbursement and regulatory processes.

ACPIs fast-track patient access to innovation, addressing challenges like regulatory uncertainty and evidence requirements; however, ACPIs vary by country. Understanding the differences between countries is crucial for manufacturers and policy makers navigating market access.

While ACPIs accelerate adoption, long-term success depends on continuous collaboration. Policy makers must refine regulatory frameworks, and manufacturers must align strategies with evolving requirements. Without adaptation, even the most promising innovations may struggle to reach patients.

The Future of Digital Health Adoption

The rapid rise of digital health technologies (DHTs), such as therapeutic digital solutions and remote patient monitoring solutions, is reshaping healthcare, personalizing treatment, expanding access, and improving patient outcomes. However, despite their potential, these innovations often struggle to fit within traditional reimbursement structures, creating barriers to widespread adoption. To bridge this gap, several countries have introduced accelerated coverage pathways for innovation (ACPIs), designed to fast-track market access for cutting-edge healthcare solutions.

> Accelerated coverage pathways for innovation offer significant benefits to patients by expediting access to groundbreaking therapies and diagnostics.

ACPIs function as early access programs, facilitating the reimbursement and integration of emerging medical technologies, including DHTs, in vitro diagnostics (IVDs), and novel therapeutics.[1] By addressing clinical and economic uncertainties, these pathways support healthcare providers in delivering innovative solutions to patients faster and more efficiently.

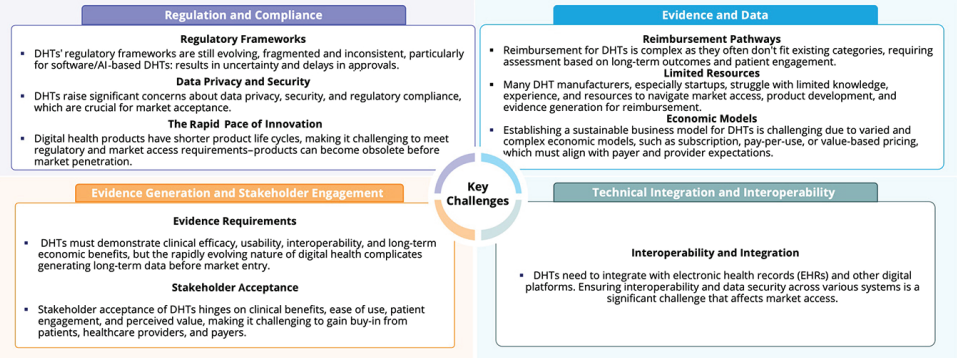
This article explores how leading ACPIs across Europe, such as the United Kingdom's AI in Health and Care Award, Germany's DiGA, and France's PECAN, are shaping the digital health landscape, their impact on market access, and the challenges that remain in ensuring sustainable adoption.

The Market Access Challenge: Why DHTs Need ACPIs

Despite the promise of DHTs, their integration into healthcare systems remains an uphill battle. Unlike traditional medical devices, DHTs face several challenges, such as regulatory uncertainty, interoperability issues, and stringent data privacy requirements. The fast-paced nature of digital innovation only adds to the challenge; by the time a technology clears reimbursement hurdles, it risks becoming obsolete (**Figure 1**).

These complexities underscore the necessity of ACPIs. Early access pathways are tailored to DHTs, as they are designed to provide structured yet adaptable solutions, offering a lifeline for cutting-edge digital health solutions seeking market entry. By streamlining

Figure 1. Graphic demonstrating the key challenges faced by digital health technologies for integration in various health systems.



DHTs, digital health technologies.

reimbursement and regulatory processes, these pathways ensure that groundbreaking technologies don't just remain theoretical advancements but reach the patients who need them most. These pathways provide temporary funding for solutions that are not yet mature enough for long-term reimbursement and require additional evidence generation. They are generally selective, with varying eligibility criteria based on factors like development stage and CE marking.

Fast-Track to the Future: How Europe Is Funding DHTs

When it comes to accelerating the adoption of digital health technologies, Germany, France, and the United Kingdom (UK) are leading the way.

Digital Health Applications (**DiGA**, Germany) focuses on low-risk digital health applications (Class I or II under European Medical Device Registration), allowing 1 year of manufacturer-set reimbursement while companies gather clinical and economic evidence to demonstrate positive healthcare effects for permanent listing.[4,5]

La prise en charge anticipée numérique (**PECAN**, France) has a broader eligibility scope, covering remote patient monitoring and higher-risk medical devices (Class I-III). It provides temporary coverage (set by the Ministry) for 1 year, giving manufacturers time to generate the necessary clinical and economic data for permanent reimbursement.[2]

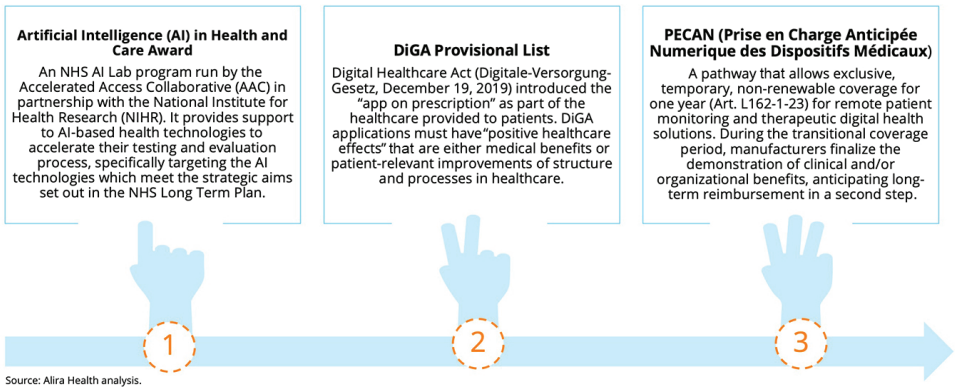
Artificial Intelligence (AI) in Health and Care Award (UK) directly funds technologies across multiple development phases, from feasibility studies to large-scale adoption. It also supports devices with market approval (MA) that require further evidence for full integration into the healthcare system.[3]

Each pathway serves as a launchpad for innovation, enabling manufacturers to secure temporary coverage and build the necessary evidence base for long-term reimbursement and adoption.

A Closer Look at Europe's Leading ACPIs

A comparative analysis of 3 major digital health–focused ACPIs (AI in Health and Care Award [UK], DiGA [Germany], and PECAN [France]) reveals their unique approaches to facilitating DHT adoption (**Figure 2**).

Figure 2. The 3 accelerated coverage pathways for innovation used in this analysis. They were chosen based on their pathway designs for digital health technologies adoption in EU5 health systems.



Source: Alira Health analysis.

AI, artificial intelligence; NHS, National Health Service.

Table 1. Applicability of the 2 digital health technologies analyzed on the eligibility criteria of the 3 accelerated coverage pathways for innovation.

Countries	Eligibility Criteria Considered	Device 1 – Non-invasive Measurement of a Biomarker for Remote Patient Monitoring	Device 2 – Tailored Therapeutic Exercises to Relieve Pain
DE FR	CE mark	X	√
DE	Class of device (I/IIa)	√	√
DE FR UK	Available evidence of the medical benefit or of a positive organizational impact	√	√
DE FR UK	Not funded by another pathway	√	√
DE FR UK	Innovative characteristic (clinical or organizational)	√	√
DE	Main function achieved through digital apps	√	√
DE FR	Validated technical requirements (eg, data protection, interoperability, etc)	X	√
UK	Use of AI	√	√
DE	The solution is not only about remote patient monitoring	√/X	√

AI, artificial intelligence.

While all innovative pathways for DHT adoption in the 3 countries aim to expedite the delivery of potentially life-saving technology, each targets different types of solutions and stages of development.

- The UK's AI in Health and Care Award demonstrated the highest theoretical eligibility (100%), followed by Germany and France (50% each). This suggests that the UK's system is the most accommodating for DHTs at various development stages.
- Unlike AI in Health and PECAN, Germany's DiGA pathway does not strongly emphasize remote patient

monitoring (RPM), potentially limiting the adoption of RPM-dependent solutions. However, with AI-driven RPM gaining traction (eg, RPM for diabetes patients, oncology patients, and in patients with chronic diseases in general), future adjustments to DiGA may integrate more such technologies.

Overall, the eligibility criteria are similar across the 3 pathways. The main differences would be in the requirements of CE marking and the integration of AI (**Table 1**). The PECAN pathway is still evolving, with only 1 device assessed under the program at the time of this analysis.

Pathway Perspectives: The Manufacturer's Dilemma

For manufacturers of DHTs, securing reimbursement is often as complex as developing the technology itself. Success hinges on meeting rigorous clinical and economic benchmarks, navigating country-specific regulations on safety, AI, and data privacy, and crafting a market strategy that aligns with evolving funding opportunities. But with each country applying its own rules, the landscape is anything but uniform.

> Policy makers hold the key to making accelerated coverage pathways for innovation a driving force in healthcare innovation.

Germany, for example, enforces strict safety and operability requirements, particularly for later-stage therapeutic devices, while the UK takes a more flexible approach, focusing on earlier-stage AI-driven innovations without mandating a CE mark. Programs like the AI in Health and Care Award stand out by offering comprehensive support, funding, National Health Service integration, and real-world evidence generation, helping manufacturers bridge the gap from development to adoption. Meanwhile, PECAN in France and Provisional DiGA in Germany provide temporary funding, allowing companies to introduce their products while refining clinical evidence.

These pathways don't just lower financial risk for manufacturers; they also accelerate patient access to potentially life-changing technologies.

Fast-Track Access: A Win for Patients

Beyond industry stakeholders, ACPIs offer significant benefits to patients by expediting access to groundbreaking therapies and diagnostics. Programs like the AI in Health Award involve patients in early stage real-world evidence generation, ensuring that life-changing technologies reach them sooner.

Similarly, DiGA and PECAN provide transitional coverage, allowing patients to access treatments while long-term reimbursement decisions are pending. In Germany, the Provisional DiGA model enables manufacturers to set interim prices—generating revenue while collecting additional clinical data. Meanwhile, PECAN serves as a bridge to France's broader reimbursement system, facilitating faster integration into standard care.

These 3 programs enable patients to access the technology earlier, offering quicker relief and improved outcomes for those who need it most.

Policy Implications: Driving Healthcare Transformation

Policy makers hold the key to making ACPIs a driving force in healthcare innovation. By streamlining reimbursement processes, they don't just accelerate the adoption of DHTs, they also create a ripple effect across the entire healthcare ecosystem. More efficient coverage pathways lead to long-term cost savings, particularly in managing chronic diseases, where early intervention can prevent expensive complications. At the same time, automation and AI-driven solutions free up critical healthcare resources, allowing professionals to focus on complex cases rather than administrative burdens.

Beyond immediate efficiencies, ACPIs also fuel the growth of the digital health sector, encouraging investment in emerging technologies that have the potential to redefine patient care. A well-structured ACPI strategy doesn't just support innovation, it future-proofs healthcare systems, ensuring they remain adaptable, responsive, and ready for the next wave of technological advancements.

Looking Ahead: The Future of ACPIs

DHTs present a unique challenge, balancing rapid innovation with regulatory rigor. While ACPIs provide an essential bridge for early adoption, their long-term impact remains uncertain due to selective eligibility and temporary coverage models. As healthcare systems mature, more permanent and streamlined assessment processes will likely emerge.

Ultimately, manufacturers, patients, and policy makers must collaborate to refine these frameworks, ensuring that digital health innovations not only reach the market but also sustain long-term impact in patient care.

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Reconciling the Irreconcilable: France's Approach to Balancing Drug Prices and Patient Access

Interview With Virginie Beaumeunier, President, Economic Committee for Health Products (CEPS), France

Q&A

Virginie Beaumeunier brings deep expertise in competition law and economic regulation to pharmaceutical pricing. Her background, including roles at the Finance Inspectorate and Competition Authority, shapes her approach to balancing innovation, cost control, and industrial sovereignty. This interview highlights France's evolving regulatory landscape, marked by a shift toward evidence-based pricing, greater predictability, and consideration for environmental and industrial policy goals.

"My mandate centers on reconciling what might appear irreconcilable: maintaining public expenditure discipline while ensuring patient access to innovative treatments under sustainable reimbursement conditions."

– Virginie Beaumeunier

PharmaBoardroom: Could you begin by outlining your primary mandate and vision for CEPS during your first 5 months in this role?

Virginie Beaumeunier: My appointment represents a convergence of economic regulation expertise with healthcare policy imperatives. I have cultivated a background in competition and regulatory economics, particularly through my leadership of the General Directorate of Competition, Consumer Affairs and Fraud Prevention—itself a CEPS member organization. My experience at the Competition Authority as Rapporteur General provided valuable exposure to healthcare sector dynamics, including contentious generic medicine cases and comprehensive studies on pharmaceutical distribution networks.

The pharmaceutical landscape has undergone remarkable transformation over the past 3 decades. Previously, we encountered relatively few innovative products, and even the most advanced therapeutics carried costs that appear modest by contemporary standards. Today, we observe not only an acceleration in novel product introductions but also increased complexity through multiple indication extensions—a phenomenon that presents particular regulatory challenges.

My mandate centers on reconciling what might appear irreconcilable: maintaining public expenditure discipline while ensuring patient access to innovative treatments under sustainable reimbursement conditions and simultaneously encouraging sovereignty and industrial development.

PB: How do you address the structural challenges inherent in France's segmented healthcare system?

VB: The siloed functioning of different healthcare sectors—between pharmaceuticals and medical devices, hospital and community care, and various health professionals—represents a fundamental impediment to coherent policy development. This fragmentation complicates the pursuit of holistic system approaches, with few stakeholders maintaining comprehensive perspectives on healthcare delivery.

Industry advocates legitimately argue for broader evaluation frameworks when pharmaceuticals reduce hospitalization durations or prevent admissions entirely. We observe compelling examples in diabetes management, where innovative medical devices complement evolving pharmaceutical interventions. Similarly, emerging obesity treatments generate claims regarding broader morbidity impacts that extend beyond traditional pharmaceutical assessment boundaries.

However, our regulatory framework operates within annual Social Security financing legislation constraints, necessitating what may appear as occasionally abrupt regulation while accommodating demands for broader therapeutic impact recognition. This tension requires evidence-based evaluation approaches. Drawing from my competition law background, I emphasize the necessity for robust demonstration of positive effects or externalities claimed for any product, whether pharmaceutical or medical device.

PB: What role does health economics play in your pricing methodology evolution?

VB: Health economics provides a comparative framework for product evaluation that, when comprehensively applied, can accommodate positive externalities while maintaining analytical rigor. This approach presents implementation challenges but offers potential convergence opportunities for our diverse objectives.

We are collaborating with the High Authority for Health (HAS) to enhance our advisory processes, ensuring that evaluations better serve our respective institutional needs. This represents a critical instrument for fostering convergence among competing priorities. However, progress requires substantive evidence rather than unsupported assertions or claims.

Another priority involves enhancing predictability for enterprises—a legitimate industry concern given our annual pricing review cycles. Companies understandably struggle with unanticipated revenue reductions, creating explanatory difficulties for French subsidiaries within international corporate structures. While we cannot abandon pricing adjustments—they reflect natural market dynamics where competition and volume increases typically reduce prices—we must improve our approach to this regulatory simulation of market forces.

PB: How do you balance international price competitiveness with domestic budgetary constraints?

VB: Industry representatives frequently emphasize European price comparisons, noting that French prices rank approximately 10% below European Union (EU) averages. Our pricing criteria incorporate European reference pricing from 4 countries—Germany, United Kingdom, Italy, and Spain—with ASMR4 products specifically referenced to the lowest European price, naturally positioning France at the bottom for these categories.

However, actual pricing reality differs significantly from published list prices, with confidential rebates affecting numerous products. In France, rebates apply to 6% of products but represent substantially higher expenditure proportions, approaching EUR 10 billion against EUR 30 billion total social security pharmaceutical expenditure for 2024. This creates information asymmetries, since in Germany, Italy, and Spain, we may have visibility on list prices, but the actual transaction prices remain unknown.

> The siloed functioning of different healthcare sectors–between pharmaceuticals and medical devices, hospital and community care, and various health professionals–represents a fundamental impediment to coherent policy development.

All countries, including the United States, negotiate confidential rebates, which makes it difficult to assess true international price competitiveness. However, we are increasingly hearing from laboratories that French list prices are being used as reference points—not only by smaller or less well-resourced countries, but potentially even within the framework of US public programs like Medicare and Medicaid or China. While there remains a significant gap between political announcements and actual implementation in the United States, the possibility that French list prices could influence reimbursement benchmarks abroad adds a layer of complexity to our own pricing strategy. It reinforces the importance of maintaining a coherent, evidence-based approach that anticipates downstream effects in global markets.

That said, we must be pragmatic. France needs reliable access to therapeutic innovation and operates in a globally competitive environment. We recognize the industry's call for geographic price differentiation and are open to it—where it is justified by therapeutic value and broader policy considerations. The challenge lies in striking the right balance: encouraging innovation and maintaining industrial competitiveness, while ensuring long-term sustainability and equitable access.

PB: Timelines for market access continue to be a source of concern. What steps are being taken to accelerate the overall process?

VB: Negotiations can sometimes drag on unnecessarily—even for marginal price differences. In many cases, the process is prolonged because laboratories begin with pricing expectations that are not aligned with the actual therapeutic value of the product. This often leads to a counterproductive dynamic, where the CEPS responds with very low offers in return, and both parties become stuck in a cycle of unproductive rounds.

At times, we see price gaps of up to 100-fold between the initial industry proposal and our first offer, which clearly signals a breakdown in realistic positioning.

A more constructive approach, based on transparency and mutual trust, is essential. Encouragingly, some companies are already adopting more pragmatic starting points, aligned with CEPS's published methodologies and legal criteria. Our ambition is to streamline discussions and avoid unnecessary rounds. There is no intrinsic value in conducting 25 iterations when a fair and balanced outcome can be reached in 5 or 6. Faster negotiation should not be mistaken for a lack of rigor; rather, it reflects maturity in the process.

> We are increasingly hearing that French list prices are being used as reference points–not only by smaller or less well-resourced countries, but potentially even within the framework of US public programs like Medicare and Medicaid.

Equally important are the delays that follow once an agreement is reached. The current timeline from signature to official publication—sometimes extending up to 3 months—is unacceptable. These lags are largely due to internal administrative procedures and ministerial approvals. To address this, we are in the process of modernizing our information systems, which are currently outdated. This overhaul should significantly improve internal coordination and reduce unnecessary bottlenecks. Ultimately, our objective is clear: regulatory needs must not stand in the way of timely patient access to treatment.

PB: How do you manage the tension between innovation rewarding and budget management?

VB: Our challenge involves maintaining access to mature but essential products—such as antibiotics or various psychotropic medications—while providing appropriate innovation incentives. Innovation often benefits limited patient populations initially, with oncology indication extensions exemplifying this pattern through highly restricted mutation-specific approvals that subsequently expand.

The challenge emerges when volume increases complicate downward price renegotiation. We must ensure mature product availability under economically sustainable conditions for both industry and social security while providing reasonable innovation access. However, innovation rewards must remain proportionate, particularly given indication extension strategies where logical volume-price relationships should apply.

We are exploring enhanced product lifecycle visibility through predetermined review schedules established at initial inscription, potentially incorporating 2-year pricing reviews based on objective criteria with guaranteed stability periods. Current stability periods exist theoretically but lack clarity and contain numerous exceptions, reducing predictability for laboratories.

PB: What innovative approaches are you developing for advanced therapy medicinal products?

VB: Article 54 provides new frameworks for innovative therapy medicinal products, particularly gene and cell therapies characterized by potentially single-administration protocols with enduring effects, personalized production requirements, and substantial manufacturing costs.

We are implementing "pay-for-performance" mechanisms involving initial forfeit payments at administration followed by patient monitoring protocols. If products deliver promised single-treatment cures, we require verification over extended periods—2, 5, or 10 years—necessitating practitioner collaboration for patient registries and monitoring systems.

The framework incorporates fractional payments with defined cessation criteria for treatment failures, alternative therapy requirements, or patient mortality. This represents sophisticated performance-based contracting requiring careful data protection protocols given sensitive health information handling requirements.

PB: How do you envision environmental considerations influencing pharmaceutical regulation?

VB: Environmental considerations present legitimate long-term concerns requiring immediate foundational work. Localization preference partly addresses carbon footprint reduction through transport minimization, though complete domestic production chains remain rare.

The most effective environmental measure involves consumption reduction—the least polluting medicine is the unconsumed one. This emphasizes prevention, appropriate usage, and waste elimination. When patients request excessive quantities that remain unused, we witness direct environmental and economic inefficiency.

Industry production and packaging improvements deserve recognition, though we must carefully consider whether pharmaceutical pricing should accommodate additional environmental objectives. Our pricing instrument already addresses multiple targets—expenditure control, access provision, sovereignty, and reindustrialization—potentially risking to reach none of them.

Alternative mechanisms, such as reimbursement prioritization for products with superior carbon profiles within therapeutic classes, might prove more effective than pricing adjustments. We must avoid overburdening our primary regulatory instrument while maintaining environmental progress.

PB: What digital transformation initiatives are you pursuing?

VB: Our digital evolution encompasses multiple dimensions. Immediate improvements include laboratory portal enhancements and internal database modernization, improving staff efficiency and processing timelines. Production implementation targets next year.

Advanced therapy medicinal products require sophisticated data transmission and registry management systems. More broadly, we are exploring real-world evidence integration for product performance assessment, extending performance-based contracting concepts beyond innovative therapies to traditional pharmaceuticals.

> Our improvement opportunity lies in more holistic, comprehensive health system approaches.

France possesses substantial health data resources that remain fragmented across individual hospital and research teams with limited interconnectivity. While data protection vigilance remains appropriate, we might benefit from reduced proprietary system approaches and enhanced collaboration between laboratories, hospitals, and research teams. The Health Data Agency should address these challenges, particularly given artificial intelligence opportunities for enhanced clinical studies.

PB: What message would you convey to international pharmaceutical stakeholders regarding France's attractiveness?

VB: France maintains exceptional clinical research capabilities contributing significantly to international attractiveness. Despite financial challenges, our social protection system provides unique global solvency guarantees. Although our global market share continues to decline, we remain committed to innovation and attractiveness promotion.

Our improvement opportunity lies in more holistic, comprehensive health system approaches. Widespread stakeholder willingness exists; we must now transition from conceptual discussion to practical implementation. The foundation exists for meaningful progress in this direction.

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