

JANUARY/FEBRUARY 2020 VOL. 6, NO. 1

# VALUE & OUTCOMES SPOTLIGHT

*A magazine for the global HEOR community.*

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# All data are not created equal. Understanding and predicting outcomes and costs requires specialized data.



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Arthritis (RA)



Heart Failure (HF)



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# VALUE & OUTCOMES SPOTLIGHT

JANUARY/FEBRUARY 2020  
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The mission of *Value & Outcomes Spotlight* is to foster dialogue within the global health economics and outcomes research (HEOR) community by reviewing the impact of HEOR methodologies on health policy and healthcare delivery to ultimately improve decision making for health globally.

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of the International Society for Pharmacoeconomics  
and Outcomes Research (ISPOR).*

## FROM THE EDITOR

**W**ith the proliferation of wearables, sensors, and digital technologies, the potential to collect health data directly from patients has grown exponentially. The clinical trial community is addressing these new modes of data capture head on—the US Food & Drug Administration has issued recommendations and released the “MyStudies” app, and the Clinical Trials Transformation Initiative has published guidance on “decentralized” clinical trials and use of mobile technologies for data capture. Curiously, while connected devices naturally permit collection of data in the real world, the real-world research community has been slow to appreciate digital technologies as a source of real-world data on par with administrative claims or electronic health records.

This is unfortunate, as the real-world setting has long functioned as a laboratory for clinical trial innovation, as is playing out again with digital health technologies. Surveys suggest that life sciences companies—sensing opportunities to streamline their clinical development programs—are eager to adopt virtual approaches, but their enthusiasm is tempered by perceived risks associated with unproven technologies and lack of regulatory precedents. So, instead of innovating in their pivotal clinical trials, they are turning to non-interventional research, such as real-world registries, to test out novel digital technologies. It’s also the case that virtual approaches can unleash the “power of the patient” by bringing the research process to patients rather than forcing patients to bring themselves to the research process. In short, there are many reasons why our Society should be embracing digital health technologies in real-world research.

This issue of *Value & Outcomes Spotlight* has a considerable amount of content devoted to the “digital healthcare ecosystem,” as it’s called in our featured article, which describes reasons why industry has been slow to adapt, outlines concerns for patient privacy, and suggests the need to realign resources and skillsets. This is followed by an infographic page on sustainability and capacity building in the new digital age, contributed by the ISPOR Student Network, and an article on challenges associated with electronic recruitment and validation of patients in prospective outcomes research studies.

In addition to the digital-themed content, we include a variety of material of relevance to our Society. There are articles introducing real option valuation of medical technologies and an alternative approach to patient matching in oncology studies, prioritizing the line of treatment. Our ISPOR Central section features an overview of the 2024 update to the ISPOR strategic plan, provided by CEO and Executive Director Nancy Berg. There is also a report on health technology assessment and health policy specific to the Latin American region, based on a meeting that took place last fall at the ISPOR Latin America Conference in Bogotá, Colombia.

Happy reading!



David Thompson, PhD  
Editor-in-Chief,  
*Value & Outcomes Spotlight*



**ISPOR SPEAKS**

**Transformation: At the Heart of ISPOR Strategy Into 2024**

Nancy S. Berg, CEO and Executive Director, ISPOR

As many of you already know, much of the work of ISPOR's 2020 Strategic Plan was completed ahead of schedule, which was no easy feat as that work was significant. Here are just a few highlights of what was accomplished in the past few years:

- We successfully expanded ISPOR's focus and brand from "pharmacoeconomics" to "health economics and outcomes research," thereby extending the reach and impact of the organization.
- We developed and implemented new communication strategies, including a new logo and branding, and completely redesigned our website to include comprehensive content features geared at both science and nonscience audiences. We published 2 editions of the "ISPOR Top Ten HEOR Trends" and a new annual report.
- During the last strategic plan implementation, ISPOR was reshaped with new governance to support stakeholder expansion and to better serve the exponential growth of ISPOR member groups.
- New programs and events were launched, strengthening regional relevance and global education outreach. In support of these mission-critical initiatives, we dedicated millions of dollars year over year to support education and groups in lower- and middle-income countries. New Task Force Reports and white papers were published, and special interest groups were formed to address strategically prioritized topics such as real-world evidence and health technology assessment.
- Our journals grew in prominence and serve as significant global publications in the field of health economics and outcomes research (HEOR).

- We initiated business plans to expand into the medical device sector, a growing part of our membership, and launched a digital transformation plan to help us prepare for the rapid growth in technology and data, particularly around the use of real-world evidence, artificial intelligence, and machine learning.
- ISPOR opened dialog with more stakeholder groups, including payers, patient engagement and health professional organizations, and international and supranational organizations. And the ISPOR awards program evolved to better spotlight the importance of HEOR globally.

**ISPOR Strategic Plan Update 2024**

The ISPOR Strategic Plan Update 2024 builds on our previous Strategic Plan and is anchored around 4 strategic pillars and important operational values (see Figure). The Society's mission, vision, and its strategic pillars remain the same. ISPOR exists as a scientific and educational society to promote health economics and outcomes research excellence to improve decision making for health globally.

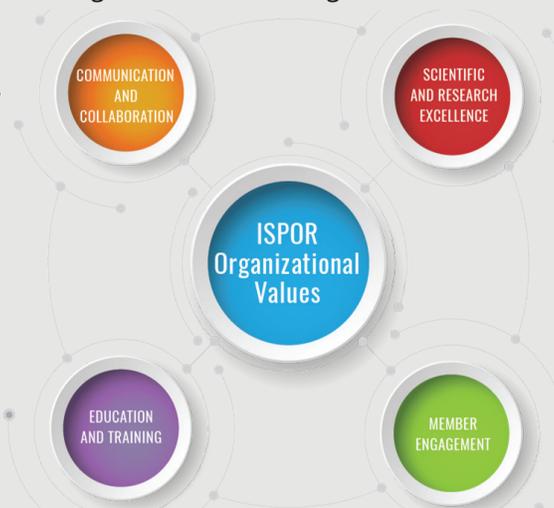


In our recent planning process, ISPOR placed transformation at the heart of its new 5-year strategy. In the updated plan, we clarified our view of the future of HEOR and its use in healthcare decision making in a white paper *HEOR's and ISPOR's Impact on Healthcare Systems and the Multistakeholder Community*. This paper describes the expanding role of HEOR across global healthcare systems.

The updated strategic plan identifies and describes ISPOR's primary objectives for the coming years. The following bulleted points highlight a few of the Society's key initiatives and areas of focus:

- Define best research practices in the science of HEOR and promote their use to improve healthcare decision making globally.
- Engage members and stakeholders to drive excellence in our programs, publications, and activities to support networking and professional growth.
- Lead efforts to strengthen and expand capabilities in HEOR. Knowledge and skill building are at the core of the ISPOR mission.
- Communicate the value and enhance the impact of HEOR. >

**Figure 1. ISPOR's Strategic Pillars**



## ISPOR CENTRAL

- Embrace a core set of values that are at the center of our vision and mission.

The Strategic Plan Update is the result of a collective effort that drew upon the diversity of ISPOR talents. Thank you to the Strategic Plan Work Group; its chair, Bill Crown, from Optum; and to the ISPOR Board of Directors for its outstanding work in moving forward a robust plan update that will elevate the importance of both HEOR and ISPOR.

We have made a great deal of progress and our strategic emphasis remains focused and innovative. In the coming

months, you will notice greater emphasis around more effectively engaging members. We encourage you to get involved—talk to a board member or staff or visit the [Get Involved](#) page on our website. We are particularly interested in hearing from mid-career professionals as we advance our diversity agenda.

There will be increased communication around how ISPOR is making an impact through good research practices. You may recall that last year we surveyed payers and decision makers to identify their level of awareness of ISPOR and their use of our papers and other

tools. We expect more of that type of engagement in 2020 and beyond.

In addition, we will be increasing communication to all stakeholders to ensure that our mission is aligned with the rapid changes taking place around the world. Specifically, ISPOR will accelerate its collaboration with payers and other decision makers.

This is an exciting time for HEOR professionals and for all of us at ISPOR. I am proud to be part of the Society's ongoing transformation! •



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[ispor.org/get-involved](https://www.ispor.org/get-involved)

## HEOR NEWS

**1 What Is the Status of Research on Low-Value Care?**

(Health Affairs)

Elizabeth L. Cope and Paul Armstrong summarize what is happening in research on low-value care, looking at what has been investigated by The Research Community on Low-Value Care. The community is a joint initiative of AcademyHealth, the ABIM Foundation, and the Donaghue Foundation. The summary includes who among publicly and privately funded groups is leading low-value care research; the aims of low-value care research; and the health conditions of interest in low-value care research.

<https://www.healthaffairs.org/doi/10.1377/hblog20200106.99070/full/>

**2 ICER Posts Draft Scoping Document for the Assessment of Treatments for Beta Thalassemia** (ICER)

The Institute for Clinical and Economic Review (ICER) in January posted a draft-scoping document outlining a planned review of the comparative clinical effectiveness and value treatments for LentiGlobin (Bluebird Bio) and luspatercept-aamt (Reblozyl, Acceleron Pharma Inc and Bristol-Myers Squibb/Celgene) for the treatment of beta thalassemia. Following the public comment period, a revised scoping document will be posted on or about February 4, 2020.

<https://icer-review.org/announcements/icer-posts-draft-scoping-document-for-the-assessment-of-treatments-for-beta-thalassemia/>

**3 The Most Valuable Pipeline Drugs for 2020**

(Managed Healthcare Executive)

Some of the drugs named in this report include: (1) Eli Lilly's Reyvow (lasmiditan) for acute treatment of migraine with or without aura; (2) Novartis' Adakveo (crizanlizumab-tmca) for sickle cell pain; (3) Merck's Ervebo, the first FDA-approved vaccine for the prevention of Ebola virus disease.; and (4) Alnylam Pharmaceuticals' Givlaari (givosiran) for acute hepatic porphyria.

<https://www.managedhealthcareexecutive.com/news/most-valuable-pipeline-drugs-2020>

**4 The 4 Biggest Pharma Market Access Stories of 2019**

(Pharmaphorum)

Articles on (1) biosimilars, (2) the resolution of the conflict between National Health Service and Vertex on Orkambi (lumacaftor/ivacaftor) pricing and access, (3) the ongoing US debate about drug and healthcare pricing, and (4) executive shakeups for the FDA and NICE were identified as the 4 top stories in 2019 in the market access field.

<https://pharmaphorum.com/views-analysis-market-access/the-4-biggest-pharma-market-access-stories-of-2019/>

**5 Should Access to Life-Saving Medicines Be Determined by Economic Evaluations?** (The Hill)

Gunnar Esiason, who has been living with cystic fibrosis since he was diagnosed at the age of 2, writes about his experiences with the disease and how Vertex's drug Trikafta (elexacaftor/tezacaftor/ivacaftor and ivacaftor) saved his life when he was in the end stages of cystic fibrosis. Son of Boomer Esiason, who started the Boomer Esiason Foundation to advocate for the cystic fibrosis community, Gunnar asks whether the QALY should be used for patients like himself to limit their access to innovative drugs such as Trikafta, calling the economic model "discriminatory."

<https://thehill.com/opinion/healthcare/477547-should-access-to-life-saving-medicines-be-determined-by-economic#.Xhd8lzMRROU.twitter>

**6 Trends in List Prices, Net Prices, and Discounts for Originator Biologics Facing Biosimilar Competition**

(JAMA Network Open)

In 4 case studies, the authors of this paper observed that the net prices of originator biologics decreased following the entry of biosimilars or other substitutes. While the decreasing net prices of infliximab and filgrastim had been previously described, this study is the first to examine pegfilgrastim and insulin glargine and the contribution of non-Medicaid discounts toward lowering net prices.

<https://jamanetwork.com/journals/jamanetworkopen/fullarticle/2757480>

**7 Shaping the Patient-Centric Evolution of HTA in Europe**

(Pharmaphorum)

Patient engagement is one of the most important drivers for improving healthcare delivery but, as Krystallia Pantiri explains, approaches by European health technology assessment (HTA) bodies vary.

<https://deep-dive.pharmaphorum.com/magazine/patient-engagement/shaping-the-patient-centric-evolution-of-hta-in-europe-pharmerit/>

**8 Google Cloud and FDA MyStudies: Harnessing Real-World Data for Medical Research** (Google Cloud)

According to Jameson Rogers, PhD, product manager, Google Cloud Healthcare & Life Sciences, by making FDA's open-source MyStudies platform available on Google Cloud Platform, the company hopes to "stimulate an open ecosystem that will improve the ability of organizations to perform research that leads to better patient outcomes." Google Cloud is working to expand FDA's MyStudies platform with built-in security and configurable privacy controls, and the ability for research organizations to automatically detect and protect personally identifying information.

<https://cloud.google.com/blog/topics/healthcare-life-sciences/fda-mystudies-comes-to-google-cloud> >

**9 Health Technology Assessment: Europe Cannot Afford an Inefficient System**  
(The Parliament Magazine)

UCB CEO Jean-Christophe Tellier looks at the Commission Proposal for a European Regulation, saying that a European-wide system needs to fully integrate with national processes, rather than adding a supplementary hurdle that would effectively mean delays for patients. But Teller is concerned that European Union Member States will opt for a compromise that will inevitably lead to an inefficient system of joint clinical assessments used in HTA processes.

[https://www.theparliamentmagazine.eu/articles/partner\\_article/efpia/health-technology-assessment-europe-cannot-afford-inefficient-system?utm\\_campaign=Industry+news+&utm\\_content=twitter&utm\\_medium=social&utm\\_source=twitter](https://www.theparliamentmagazine.eu/articles/partner_article/efpia/health-technology-assessment-europe-cannot-afford-inefficient-system?utm_campaign=Industry+news+&utm_content=twitter&utm_medium=social&utm_source=twitter)

**10 Do Market Access Withdrawals Impact Patient Access to Treatment in Germany?** (PRMA Consulting)

PRMA Consulting did a study of negotiations between manufacturers and the GKV-Spitzenverband to understand the number and timing of drug withdrawals and their impact on supply to patients. While the study shows that several pathways exist for continuing supply to German patients, treatment disruptions due to delayed price agreements or re-introductions are still likely.

<https://www.pрмаconsulting.com/market-access-publications/Market-withdrawals.pdf>

**11 Cost-Effectiveness and Cost-Utility Analysis of a Work-Place Smoking Cessation Intervention With and Without Financial Incentives** (Society for the Study of Addiction)

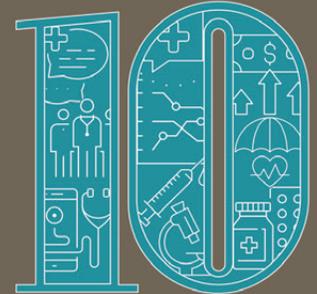
An economic evaluation conducted at 61 companies in The Netherlands examined a work-place smoking cessation group training program with incentives compared with a training program without incentives. In their analysis, the authors concluded that while financial incentives added to a smoking cessation program does increase initial costs, the increase in the number of quitters could improve the cost-effectiveness in the future through better employee health, making financial incentives a short-term investment that pays off.

<https://onlinelibrary.wiley.com/doi/full/10.1111/add.14861>

**12 IVI's Updated Rheumatoid Arthritis model Examines New Treatment Options, Treatment Effects and Cost Estimates** (Innovation and Value Initiative)

Innovation Value Initiative (IVI) released its updated rheumatoid arthritis model in January, which now incorporates additional treatment options (triple therapy, Janus Kinase (JAK) inhibitors, sarilumab, and biosimilars), updated treatment effect estimates based on additional randomized controlled trial evidence, and updated cost estimates. The model is also designed to answer a variety of critical questions for patients, payers, and providers if given real-world patient data as input. The organization is seeking data partners.

[https://www.thevalueinitiative.org/wp-content/uploads/2020/01/2020-01-07.IVI-RA-model-update-press-release\\_FINAL.pdf](https://www.thevalueinitiative.org/wp-content/uploads/2020/01/2020-01-07.IVI-RA-model-update-press-release_FINAL.pdf)



ISPOR 2020

TOP10  
HEOR  
TRENDS

[ispor.org/  
top10trends](https://www.ispor.org/top10trends)

## CALL FOR PAPERS

### Opioid Misuse: A Global Crisis

# Value in Health

Recent trends in opioids have changed policy discussions of drug use from that of a problem—or an epidemic—to a global crisis. An estimated 27 million people suffered in 2016 from opioid use disorders. Globally, approximately 450,000 people died as a result of drug use in 2015 and about 160,000 were directly associated with drug use disorders; 118,000 dying with a opioid use disorder.

Recognizing the urgent need to address this public health crisis and the meaningful expertise that ISPOR members can make to research effective and efficient solutions, the Editors of *Value in Health* are issuing an open Call for Papers on a wide array of topics that could inform policy and healthcare decision making in solving the global opioid crisis.

Topics of interest include, but are not limited to:

- Systematic reviews of evidence on the root causes of the opioid crisis
- Qualitative and quantitative evidence describing the impact to patients, families, communities, and employers when touched by the opioid crisis
- Summary of evidence for individual- and population-level preventions of opioid misuse
- Summary of evidence for individual- and population-level treatments of opioid misuse
- Commentaries on appropriateness criteria and monitoring use of opioids
- Summary of future needs, solutions, and evidence development

Submissions received before **March 1, 2020** have the best chance of being included in this themed section. Final decisions regarding ultimate acceptance rest solely with the Editors.

Authors should submit manuscripts through the journal's web-based tracking system at <https://mc.manuscriptcentral.com/valueinhealth> and be sure to classify their submissions as Opioid Crisis themed section.

[www.ispor.org](http://www.ispor.org)

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# ISPOR 2020

May 16-20, 2020 | Orlando, FL, USA

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# ISPOR 2020 Conferences

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**March 31 | Silver Spring, MD, USA**

*Using Patient-Preference Information in  
Medical Device Regulatory Decisions:  
Benefit-Risk and Beyond*

In Person Registration: March 31  
Virtual Registration: March 31  
*Broadcast via Livestream*  
Pre-Event Webinar: March 4



**May 16-20 | Orlando, FL, USA**

*HEOR: Advancing Evidence to Action*

Abstract Notification: March 2  
Early Bird Registration by April 7



**12-15 September | Seoul, South Korea**

*Next Generation Healthcare in Asia Pacific:  
Where Technology Meets Patients to  
Improve Care*

Abstract Submission Closes: 11 March  
Abstract Notification: 1 May  
Early Bird Registration by 28 July



**29-30 September | Dubai, UAE**

*Value-Based Healthcare: Health Policy,  
Economics and Outcomes*

Early Bird Registration by 18 August



**14-18 November | Milan, Italy**

*Improving Health: Establishing Incentives  
and Sharing Value*

Abstract Submission Opens: 2 March  
Abstract Submission Closes: 10 June  
Registration Opens March

## Looking ahead to 2021 Conferences:

**ISPOR 2021**  
May 15-19  
Montreal, Canada

**ISPOR Latin  
America 2021**  
Mexico City, Mexico

**ISPOR Europe 2021**  
6-10 November  
Paris, France

[www.ispor.org](http://www.ispor.org)

## Latin America Policy Makers Grapple With Health Technology Assessment and Health Policy

Ramiro Gilardino, MD, MHS, MSc, ISPOR, Lawrenceville, NJ



ISPOR held its Latin America Regional Health Policy Summit on September 11, 2019, in Bogotá, Colombia. With policy makers, payers, industry, and patient representatives from Argentina, Brazil, Colombia, Chile, Mexico, Peru, and Uruguay, as well as guests from Asia, Europe, and the United States, the summit addressed how to translate outcomes research into policy decisions. Policy makers from the region discussed health technology assessment (HTA) as a tool for prioritizing health resources, assessing the value of novel and innovative technologies versus disruptive innovators, and the implementation of managed entry agreements in Latin American countries. Keynote presenters included Hector Castro MD, MSc, PhD, Management Sciences for Health, Washington, DC, USA; Manuel A. Espinoza, MD, MSc, PhD, Pontificia Universidad Católica, Santiago, Chile; Ramiro Gilardino, MD, MHS, MSc, ISPOR, Lawrenceville, NJ; and Jan Weinreich, PhD, Roche Pharma, Basel, Switzerland

### Current Stage of Universal Health Coverage and the Role of HTA in Designing Health Benefit Packages

Universal health coverage (UHC) aims to provide access to qualified health services and financial protection from catastrophic health expenditures. The discussion, led by Dr. Ramiro Gilardino, pointed out that while UHC is on the agenda of most countries in Latin America, and many have improved healthcare coverage through primary healthcare implementation and healthcare systems modifications, many countries are struggling to find a mechanism to expand the service coverage to people with unmet needs and to increase measures to guarantee financial protection for catastrophic expenditures.

Prioritized health services baskets, also called “health benefits packages,” have been shown to be a cost-effective method to improve the UHC index

through increasing the delivery of services (eg, pharmaceutical products, medical devices, diagnostics tests, and diagnostic/therapeutic procedures), while reducing costs that could impoverish patients.<sup>1</sup> In the discussion, participants shared that some countries in Latin America have explored this strategy as an efficient alternative that can strengthen the healthcare system.

HTA could be employed as a decision support tool to review and summarize the comparative evidence of healthcare interventions, although the lack of technical capabilities and limited experience in low- and middle-income countries have limited progress.<sup>2</sup> Recognizing that HTA is in different levels of development in Latin America, the participants said HTA has a promising role in informing what new technologies to incorporate in the health benefits packages, as well as those that are outdated and should be removed

from the benefits packages. The use of HTA for disinvestment was said to be important, but it was stated that none of the countries represented at the Summit currently use HTA regularly for this purpose.

Mimicking the ongoing European Union HTA harmonization process, a joint comparative clinical efficacy assessment is something that could be replicated in Latin American countries. But participants noted that the development of joint initiatives (ie, joint purchasing) would require the support from global organizations such as the Pan American Health Organization/REDETSAs (the Health Technology Assessment Network of the Americas). Finally, the discussion also brought to light that the lack of defined and uniform decision-making rules (eg, thresholds or explicit prioritization) is a challenge for the assessment and appraisal of high-cost drugs, especially for ultra-rare diseases and those that might be a disruptive innovator.

### Value in Health Coverage Decision Making

In the first part of this session, Manuel A. Espinoza described different approaches to measure value in health, emphasizing the challenges and opportunities for local healthcare systems to implement a systematic decision-making process for coverage and reimbursement. This presentation highlighted that in Latin America, significant efforts have been made to build capacities to characterize health benefits and reveal the value of these technologies, mostly anchored on the principles of evidence-based medicine.

Other health systems have taken a step forward, considering the opportunity cost of an alternative use of limited resources. This consideration of health benefits forgone elsewhere in the health system is revealed through cost-effectiveness analysis, a type of study that is increasingly being taken into account in Latin America.

More recently, some countries have paid attention to alternative approaches to reveal value, including general methods such as multicriteria decision analysis and evidence to decision framework,<sup>3</sup>

as well as specific instruments such as value assessment frameworks developed by scientific societies or healthcare institutions.<sup>4</sup>

### Assessing the Value of Novel and Innovative Health Technologies

As part of the second session, Jan Weinreich described that the broader understanding of all value components will, in turn, foster understanding of the societal benefits of healthcare investment resulting in increased access to medicines. This “proposed” value framework will capture a comprehensive perspective on the value of medicines for society and lay the foundation for stakeholder engagement with the ultimate objective of patients and society benefiting from the advances in science.

There is a general belief that countries in Latin America are not yet prepared to adopt innovative technologies. Additionally, external models for incorporation might not apply in the regional context; however, much of the data presented in the session, which was based on surveys of participants, demonstrated otherwise.

**In countries where health services are generally accessible and affordable, governments are struggling to respond to rising healthcare costs and the growing health needs of their populations.**

According to discussion participants, the elements of value for innovation should include cost-effectiveness and budget impact analysis as well as the societal perspective, which considers how much health the patient gains and what is the cost of that gain. For patients with cancer, the assessment of their health status requires strong outcomes measures like overall survival or progression-free survival; however, this could be difficult to obtain in patients with some types of rare cancers. Additionally, discussion participants said there is a need to standardize how the innovative technologies will be incorporated to avoid inequities and inequalities in access to health services. Acknowledging that the valuation of innovation should follow the established HTA process, harmonization between the

different HTA agencies might increase knowledge and improve the capacity to perform this kind of assessment.

When innovation provides clear value for the population, but funding constraints challenge its adoption, a value-based approach, with the support of the health benefits packages, could be implemented.

Additionally, when fragmentation and multiple financial mechanisms exist for a certain disease, prioritizing and harmonizing them into a single policy could improve the allocative efficiency and increase patient access. Surveys of the participants noted that personalized medicine would benefit a small portion of the population, roughly less than 10%. There were also mixed perceptions about how these technologies should be incorporated and funded, specifically, when the participants were surveyed to assess their thoughts on how these novel technologies should be financed, the majority of them responded “partially” when asked if the funding should come from public resources.

### Price Negotiation and Management of Entry Agreements

The discussion led by Hector Castro explored the opportunities and challenges for implementing price negotiations and managed entry agreements in Latin American countries. Barriers to and facilitators for were explored throughout the session in order to promote a policy dialogue among participants. According to Dr Castro, while unfinished agendas for infectious diseases like HIV, tuberculosis, or malaria are still existing in many low- and middle-income countries, the burden of noncommunicable chronic conditions has substantially climbed to the top as one of the most pressuring concerns in these settings.

Policymakers in many low- and middle-income countries are >

interested in combining a mixture of policy interventions in order to reach sustainable UHC, mostly by improving their levels of allocative and technical efficiency. Decision makers in low- and middle-income countries are considering a number of policy ammunition tactics for this purpose, including price negotiation of healthcare commodities and managed entry agreements. Managed entry agreements represent a potential opportunity for granting early access to innovation; however, as in the case of price negotiation, they also come with caveats including heavier transaction costs for the healthcare system.

According to the participants, financial agreements followed by hybrid schemes were the most common type of managed entry agreements seen in the region. Also, there was consensus that managed entry agreements should be a joint effort initiated by the payer (either public or private) and the technology producer. Participants stated that they believe that the challenges to the future development of managed entry agreements include lack of financial incentives (eg, pay for performance), lack of administrative and clinical data collection or strong set of data, and internal government legal barriers that would prevent timely implementation. Some participants said the fragmentation of the health systems in their countries would allow manufacturers to make different managed entry agreements, without the other sectors of the health system knowing about them. Additionally, there are manufacturer monopolies for certain drugs that would make negotiations difficult, especially in small countries. To move forward, participants said the factors needed are: (1) alignment between stakeholders and political will to commit (and trust among the stakeholders involved); (2) adequate regulatory and legal frameworks that ensure transparency of the process, including the outcomes assessment; and (3) mechanisms that favor countries that may be disadvantaged during the price negotiations (eg, consolidated purchasing).

Participants said they believe that when the regulatory and reimbursement agencies, the technology producers, the HTA, and the healthcare providers join

efforts and align, a fast-track process for the access of high-cost drugs to the patient can be achieved. Cited as an example was how Colombia handled the assessment of, and negotiations for, hepatitis C drugs.

Ultimately, according to participants, a successful agreement would need to be built on a comprehensive process that includes the patient selection, the treatment protocol, and the data collection and analysis.

ISPOR President-Elect Jens Grueger, PhD pointed out that access to innovation is a complex area and requires the expertise and collaboration of industry, health authorities, providers and society, and, of course, patients. We need to create transparency and trust so that we can build a sustainable approach.

### Acknowledgement

The presenters would like to thank Christine Truelove for compiling and summarizing their presentations in this article. •

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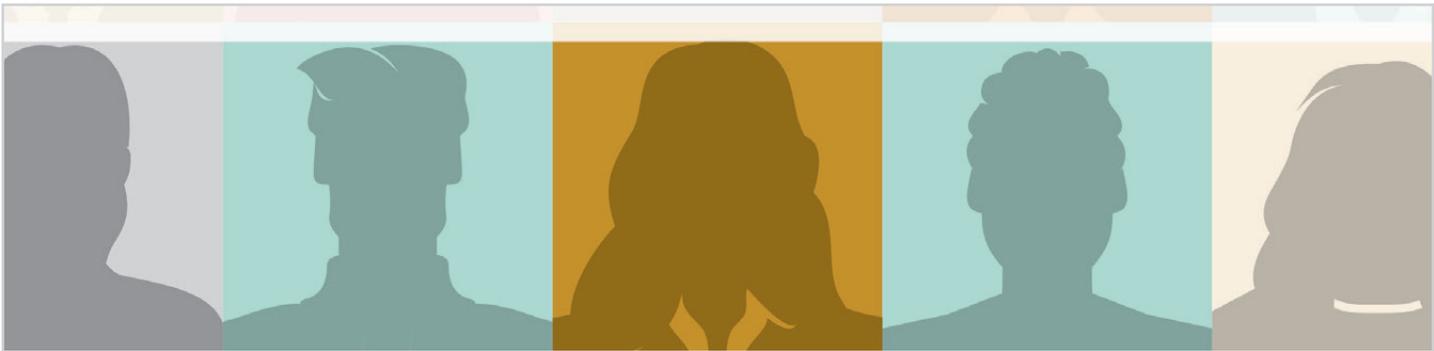
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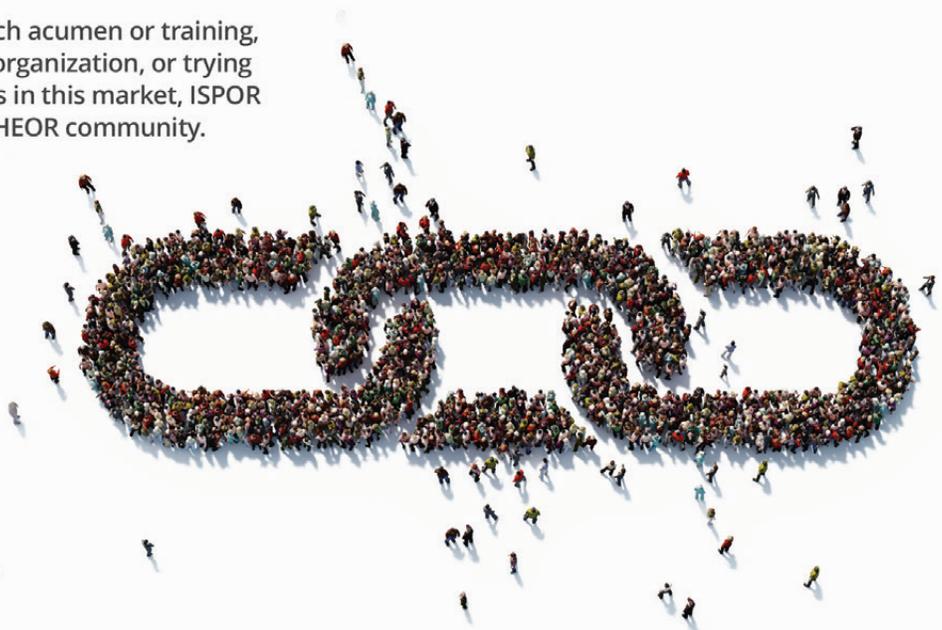


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FEATURE

# THE NEW DIGITAL HEALTHCARE ECOSYSTEM: LOOKING OUTSIDE TO HARNESS FROM WITHIN

BY MICHELE CLEARY



Navigating the digital landscape of patient-provided data—from wearables, to at-home DNA tests, to clinical data registries—grows evermore complex. In this month's feature article, experts in the field provide different perspectives on how best to leverage digital health technologies and to apply the overabundance of data to real-world research.

### **New Digital Ecosystem, New Opportunities**

We welcome this new decade facing a new digital era in healthcare. Ever-growing mountains of digital data produced from new technologies—ranging from wearables and monitors to at-home genomic tests and newly digitalized clinical data—are providing an unprecedented opportunity to improve our understanding of disease onset and progression.

These data can help refine diagnostic methods and improve treatment selection, can accelerate research and development processes by acting as external control arms or by supporting label expansion, and can facilitate targeted patient engagement programs. As Katie Szyman, Corporate Vice President of Critical Care at Edwards Lifesciences heralded, "The data/digital revolution creates a great opportunity for transformation in healthcare."

This new digital era allows us unprecedented insight into the patient experience, making these data resources especially important for value assessment. As our definition of value expands to incorporate different stakeholder perspectives, insight into not just clinical responses, but also patient criteria (eg, social determinants of health, treatment compliance behavior) become critical.

Christopher Boone, Vice President of Global Medical Epidemiology and Big Data Analytics at Pfizer, highlighted the ability of these new digital data resources to provide greater insight into the value discussion, stating, "I think the pharmaceutical industry is now in a position where it has to align its definition of value with payers, providers, and patients. That wasn't necessarily the case before, because we were essentially the purveyors of much of the data and information that was used to make those clinical decisions."

This transition requires health economic and outcomes research (HEOR) professionals to upscale their expertise. Boone continued, "In order to start to facilitate and have these discussions with these stakeholders around value and for value-based agreements, I think we are going to have to be as equally skilled in our understanding and our analysis of these data types as our stakeholders."

But where does the HEOR community stand in its journey into this new digital era?

### **Slow to Adapt?**

Despite the enormous benefits that could be delivered by healthcare's new digital world, many within the HEOR community have been slow to adapt.

This hesitancy may stem from the industry's highly regulated environment. Boone noted that he saw this hesitation in the pharmaceutical industry even 2 years ago. But in Boone's view, "It's not for lack of confidence, it's not from a lack of resources. I think it's just the byproduct of an industry that's very risk-averse."

Biotechnology and other industries that originated in information technology (IT) may help provide critical guidance. Boone cited these types of companies to play a leading role in this transformation, specifically those firms that take many of their guiding principles from legacy Silicon Valley companies. Boone noted this >

IT foundation accelerated many functions inherent to the life science industry. He stated, “These essentially digitally made companies are accelerating the R&D [research and development] process with much more robust commercialization activities and functions. They are accelerating digitization of the industry.”

Collaboration with these types of “digitally made” companies will change the nature of collaboration for many players. “I think this is an opportunity for big pharma to really learn from others rather than others learn from us,” Boone added.

### **Tackling Infrastructure Demands**

Given their origin in IT, these “digitally made” biotech companies tend to be comfortable tackling the enormous processing and analytic infrastructure demands necessary to sustain these digital data resources. Such familiarity with infrastructure challenges is critical, especially given the astonishing pace with which this digital ecosystem is growing.

## **“The data/digital revolution creates a great opportunity for transformation in healthcare.”**

Katie Szyman, Edwards Lifesciences

Currently, the volume of health data collected by providers, insurers, government, researchers, and industry is estimated to double annually.<sup>1</sup> Elean Bonfiglioli, Senior Director of Health Industry (Europe, Middle East, Africa) at Microsoft, highlighted the expansion of digital clinical data alone, stating, “Fifteen to twenty years ago, 20% of health records were digitized. Today, more than 90% are.”

Infrastructure needs mirror this explosive growth in digital data. A recent University of Michigan study estimated that the number of transistors required to process genomic and neuroimaging data increased by 2 orders of magnitude between 2014 and 2016.<sup>2</sup> And given the growing need for fast and secure data transfer, bandwidth demands are mushrooming, as well.

Many contributors highlighted the need for sound collaborative relationships as the key to tackling many of these issues. Yet despite the astounding growth in infrastructure needs, none of these contributors expressed concern that infrastructure was a primary impediment to developing a sustainable digital ecosystem for the HEOR community. Instead, they focused on more pressing concerns, such as building interdisciplinary teams of experts to improve

operations and to develop public trust in critical data-sharing operations.

### **The Importance of Interdisciplinary Collaboration**

Navigating this new digital ecosystem will require a broader range of expertise in areas such as machine learning, artificial intelligence, and other data science fields. “A big problem that the industry is facing is that the people making decisions are people without training,” said Julian Isla, CEO of Foundation29 and Professional Development Manager at Microsoft Services (Spain). “Pilots fly planes, but it would be crazy for pilots to make the planes. For that, you need engineers.”

Boone advocated strongly for interdisciplinary teams, saying, “It shouldn’t just be folks that are trained as economists. These teams are going to look much more like traditional data science teams, which have skill sets reflective of many different disciplines working in one group.”

Collaboration with data science experts may also resolve many of the interoperability challenges inherent within these new digital ecosystems, given their dependence on shared data. To operate effectively, these systems require methods that standardize data management, manipulation, data collection, and platforms that provide secure data exchange between stakeholders. Such standards are especially important, given the huge volume of unstructured healthcare data.

## **“The challenge with digitization is ensuring patients trust the system.”**

Julian Isla, Microsoft and Foundation29

The creation of effective interoperability standards for digital healthcare data is progressing. As an example, the Fast Healthcare Interoperability Resources (FHIR), developed by Health Level Seven International (HL7), promotes interoperability across the increasingly digitalized health ecosystem.<sup>3</sup> While interoperability challenges persist, interdisciplinary collaboration will help to resolve these operational challenges.

### **Building Trust in Digital Data Sharing**

The need for healthy collaboration extends beyond information technology. Given the extensive sharing of sensitive data, public views must also be considered. Bogi Eliason, Associated Partner at Copenhagen Institute for Future Studies, reflected on how central the patient becomes in the digital future, stating, “We are beginning to work more with patient-reported outcomes. The next level is to link that with different

wearables.” He sees this as leading to real patient engagement, where patients are involved as experts in their care. In other words, “The patient as part of the ecosystem.”

New digital technologies are empowering patients with the ability to organize, control, and share their healthcare information. Today, consumers desire more health information, and they want it within their control.

### Are Patients Comfortable With Data Sharing?

Historically, parties making health decisions—providers and the payers—maintained control of health data. Today ownership of these data is shifting, leading some to ask, “who actually owns the data?” More precisely, who controls where these data are sent and with whom they may be shared? Who should control the data?

## “I think this is an opportunity for big pharma to really learn from others rather than others learn from us.”

Christopher Boone, Pfizer

Boone cited his experience leading the Health Data Consortium, a public-private partnership aimed at democratizing health data, where the consensus has been that the patient owns his/her data.

Yet data sharing requires that patients trust how their data will be used. As Isla stated, “The challenge with digitization is ensuring patients trust the system. This trust may come from gleaning tangible and applicable information so that the benefits of sharing data are clearly demonstrated.”

Transparency is critical. Disclosing how these data will be used and by whom may reinforce acceptance. When patients have more understanding of what is being done with data, they may be more willing to support digital data sharing.

Public acceptance of “big healthcare data” may also be strengthened by their understanding of how their data will be used. For this, Rebecca Miksad, Senior Medical Director at Flatiron Health, proposed a shared vision between patients and digital data teams. She cited the altruism of cancer patients who express a desire to help others through their data. She stated that these patients support sharing their data to help “save someone else from having to go through what they went through...that shared vision is what enables mutual trust.”

Again, collaborative relationships with patient advocacy groups, providers, or other groups already within patients’ zone of trust, may help build public confidence in the value and security of data sharing.

Missteps could significantly impede future data sharing, greatly hindering HEOR goals. Again, strong partnerships may help avoid such potential missteps.

### Looking Ahead

This massive transformation of our health data landscape shows no signs of slowing. The new iteration of the 21st Century Cures Act proposed this past November would further expand the digital environment by supporting the development of new digital technologies and developing better methods for collecting and using real-world evidence to transform healthcare.<sup>4</sup>

This rich resource could transform patient care, but the HEOR community must evolve.

Rather than citing specific products or systems to ease the transition into this new digital healthcare ecosystem, contributors continually pointed to the importance of collaboration—partnerships across healthcare with input from IT and the public. By learning from the experiences of other industries, by engaging a broad mix of collaborators, and by building trust across all stakeholders—especially patients—we can leverage this rich data resource to truly transform patient care. •

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### Additional information

This article was based on the first two plenary sessions at ISPOR Europe 2019. Presentations from this conference can be found at [www.ispor.org/conferences](http://www.ispor.org/conferences).

### About the Author

Michele Cleary is a HEOR researcher and scientific writer with more than 15 years of experience in the healthcare field.

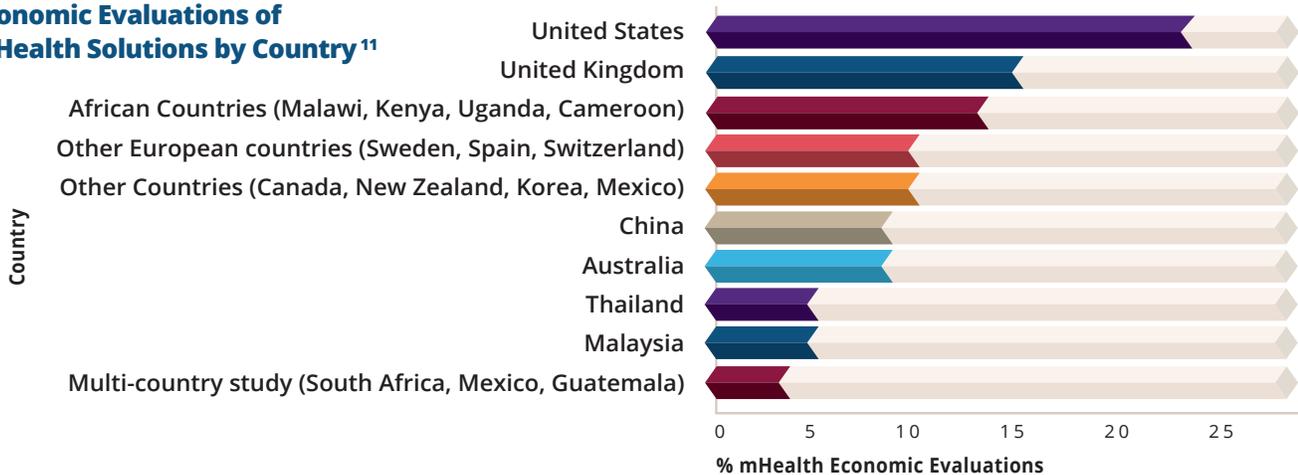
# By the Numbers: The New Digital Healthcare Ecosystem

Section Editor: The ISPOR Student Network

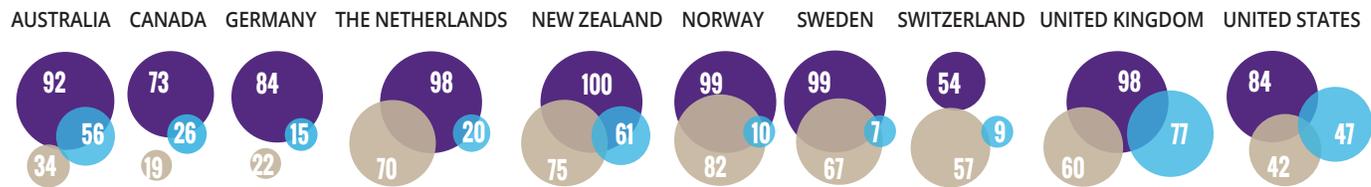
## Digital Healthcare: A Timeline

- 1993** Japanese electronics manufacturer, Panasonic™, releases a compact and automatic wrist cuff blood pressure monitor<sup>1</sup>
- 1996** Health Insurance Portability and Accountability Act passed by United States Congress that sets standards for confidentiality around personal health information<sup>2</sup>
- 1998** The Data Protection Act sets standards for maintaining confidentiality of patient records in the United Kingdom<sup>3</sup>
- 2000** The daVinci® robotic surgical system that incorporates weak artificial intelligence is approved by the US Food and Drug Administration (FDA)<sup>4</sup>
- 2004** PatientsLikeMe.com is founded to help connect patients with amyotrophic lateral sclerosis. Today, it consists of 650,000+ members across 2900 conditions representing greater than 43 million data points<sup>5</sup>
- 2007** Fitbit® founded with the vision of creating a wearable product to improve fitness and health<sup>6</sup>
- 2009** Google Flu Trends reports a potential swine flu outbreak in Mexico several weeks before clinical epidemiology system reports the same<sup>7</sup>
- 2014** A survey by Medscape reveals nearly 83% of physicians in the United States utilize electronic health records<sup>8</sup>
- 2016** A study by PricewaterhouseCoopers reveals that nearly 50% of all US consumers own a wearable device<sup>9</sup>
- 2017** The FDA releases its Digital Health Innovation Action Plan to develop approaches for the regulation of digital health products<sup>10</sup>

## Economic Evaluations of mHealth Solutions by Country<sup>11</sup>



## The Use of Health Information Technology by Primary Care Doctors in 10 Countries, 2015<sup>12</sup>



Numbers represent % of primary care doctors surveyed in each country.

KEY: ● Use an electronic medical record ● Routinely receive computerized reminders for guideline-based intervention or screening tests ● Can electronically exchange patient clinical summaries with doctors outside practice

**Contributors:** Mona Nili, West Virginia University, USA; Chintal H. Shah, University of Maryland, USA; Krystal Williams, Florida Agricultural and Mechanical University; Aakash Bipin Gandhi, University of Maryland, USA  
**Acknowledgement:** We would like to thank Zeba Khan, RPh, PhD for her review and helpful comments

References available online

# Electronic Recruitment and Validation of Patients for Outcomes Research Studies in Rare Diseases: What Are the Potential Challenges?

Monica Hadi, PhD, Evidera, London, England, United Kingdom; Joe Waby, MSc, Global Perspectives, Asturias, Spain

**For outcomes research studies in rare diseases, validation (ie, verifying patients are really who they say they are) is even more important as patients likely need to have diagnosis of a specific condition and take a specific medication for a certain period of time.**

**E**lectronic recruitment and survey completion is a popular, cost-effective way to access hard-to-reach patient groups, particularly in rare disease outcomes research. These patients are often deeply engaged with online communities regarding their condition and treatment. Patients are usually eager to discuss and share their experiences, with the intention and hope that treatments and outcomes will improve because of their participation.

Good study design for data collection from patients with rare diseases requires careful planning of the electronic recruitment and validation process ahead of time. Validation of patients for inclusion in research studies can take many forms, but essentially refers to the process of verifying that patients are really who they say they are and that they are the type of patients required for that specific study. For outcomes research studies in rare diseases, validation is even more important as patients likely need to have diagnosis of a specific condition and take a specific medication, and have been doing so for a specific period.

**Good study design for data collection from patients with rare diseases requires careful planning of the electronic recruitment and validation process ahead of time.**

To achieve realistic targets and deliver accurate and reliable data, it is important to allow for a feasibility assessment that can help tailor the study recruitment and validation process. The study team should draw up the most appropriate recruitment strategy—one that would consider factors such as the patient audience, sample size, and level of validation required. Decisions made about validation should consider how the recruitment selection process impacts patient knowledge, honesty, and engagement.

## Considerations for Sample Size and Incidence Rates

There are several challenges in recruiting large sample sizes for outcomes research studies in rare diseases. First and most obviously, patients are limited in number as incidence rates are usually very low in rare diseases, sometimes as low as 1 in 1 million people with the specific diagnosis within the general population. Often larger sample sizes are desirable to permit the use of inferential statistics and to provide more confidence overall in the conclusions drawn from the available data. To increase sample size, it might be advantageous to accept patients from sources with less-documented evidence.

Second, the way in which patients are engaged and validated as part of the study can influence whether a patient wants to take part in that study. An effective screener is needed that is tailored to the patient audience, with considerations for recruitment inclusion and exclusion criteria. The simpler the recruitment process and the less-restrictive the eligibility criteria, the easier it will be to recruit patients to a study and therefore achieve a greater sample size. However, some studies by design will necessitate more stringent eligibility criteria, which must be verified before a

patient can be screened as eligible for the study. Nevertheless, a combination of recruitment techniques and available recruitment sources is more likely to result in a larger sample size.

## Potential Sources for Electronic Patient Recruitment

Patients with rare diseases can be recruited for electronic studies from a variety of different sources, all of which have advantages and challenges as outlined in Table 1 on the following page. >

Table 1. Potential sources for electronic patient recruitment.

Recruitment Source	Advantages	Challenges
Clinical sites	Direct contact with physicians and patients allows recruitment with nearly 100% validation of eligibility.	High cost and longer timelines. Sometimes lack of willingness or engagement from sites, physicians, and patients to participate.
Physician referrals	Eligibility confirmed by a physician who can also engage the patient.	Difficult to get high numbers of patients. Depends on the country and regulations.
Patient associations	Patient associations will post information about the project and patients can register their interest in the study.	A lot depends on the patient association secretary/gatekeeper and whether they want to spend time and effort on the study.
Social media/internet support groups	Rare disease patients often use social media to help find information and support for their condition. This creates a direct link to the patient.	Need to engage patients individually. Work intensive. Low response rate. Often social media groups are closed and need administrator's permission to post.
Recruiter networks and databases	Databases of engaged patients who want to take part in studies.	The databases are not very big and can become out of date.
Patient panels	Can get high numbers of patients for rare diseases as a specialist panel has been built.	Panel owners may not reveal identity of panellists and cannot verify on telephone. Engagement may be low.
Consumer panels	Can have hundreds of thousands of panel members and therefore many rare patients.	Low engagement and inability to verify other than via general profiling.
Shared open survey links	Can get a lot of respondents quickly.	Cannot be certain that the respondents are who they say they are.

### Levels of Patient Validation

The level of validation should be determined by the study design, patient population, and recruitment source. Different levels of electronic validation may be required to ensure that patients are those the study is recruiting. Recruitment through clinical sites and physician referrals may require less-formal validation, as patients are recruited from a more reliable data source, and there is opportunity for confirmation of disease and more accurate capture of specific treatment history. Recruitment through patient associations, patient recruitment networks, or social media support groups are other popular options. Patients who join these support groups are often heavily engaged in their condition and treatment, and as a result, are often knowledgeable enough to self-confirm their validation during the screening process.

In contrast, recruitment via patient and consumer panels might pose a challenge for electronic validation, as there is little guarantee of how familiar and knowledgeable patients are about their condition. For instance, this form of recruitment might be appropriate in a study with a simple selection criterion for patients with asthma but may not be suitable for recruitment of patients with an advanced-stage cancer who may not be knowledgeable enough to self-confirm their treatment efficacy. Finally, recruitment through open survey links—even if posted on patient association or community websites or blogs—is rarely a recommended option as there is no reliable way to validate who is responding to the survey link. If this is the only available method of recruitment, the process could benefit from a detailed electronic validation procedure and further engagement with the patients to ensure they are a good fit for the study.

### Patient Knowledge

Patients may not always possess the required knowledge of their condition and treatment to self-assess their fit with the study

validation criteria. Ideally, screeners should be designed and worded in a way that patients can understand and engage with the study. There are several ways in which the study team can support patients during this process while motivating their participation. For instance, it may be beneficial for the study team to help support patients with the interpretation of technical or complicated concepts during

the validation process. This may help guide and further motivate patients to take part and share accurate information about their condition.

Some studies may require physician confirmation of disease or treatment. In these cases, the study might offer an additional incentive to patients for reaching out and acquiring confirmation of technical information from a physician. This process would rely on patients having access to their physicians in a timely manner during recruitment to gather the required information, but the process would inevitably produce more reliable data. However, it is worth bearing in mind that not all patients are willing to approach their physicians for this information, for various personal reasons. There is an important compromise between validation level and sample size. If a study requires physician confirmation of diagnosis, then the study may need to accept a lower sample size of patients than if self-confirmation of diagnosis by the patient is enough.

There should also be considerations for cultural differences among patients with rare diseases regarding disease awareness and knowledge. In English-speaking countries, patients are often engaged and knowledgeable about their condition, especially if there is opportunity for patients to select healthcare providers and treatments. This is perhaps less apparent in countries where patients are traditionally more likely to depend on their healthcare provider for information. Expending some effort in assessing the extent and reliability of patient knowledge for a given population may pay dividends in ensuring the accuracy of data obtained.

### Patient Engagement

Some electronic recruitment techniques can result in patients being less engaged in the study design and process. For studies

that source recruitment through online consumer panels, it may be that the patient respondents are used to receiving several survey requests, and as a result may not be motivated or engaged enough to complete the survey with a great deal of attention. This can lead to low-quality data (eg, speeders, flat liners), which are indicators of low engagement from patients. The study team can overcome this to some extent by aiming to engage with interested patients, highlighting the importance and value of the study for the rare disease community, and supporting them with interpretation of technical or complicated concepts. Patients with rare diseases are often part of a highly engaged community who are motivated to increase disease awareness and help encourage the availability of treatments, and this alone could yield higher patient engagement.

### Patient Honesty

Although rare, the risk of wrongful recruitment or dishonesty from the respondent's side becomes an issue when using online open survey links that do not involve human or profiling validation steps. This increases the chance of a "fake" patient being involved, who may not have a diagnosis of the rare disease in question but may be interested in the offered patient incentive. It is important that survey access is limited to those who already have been electronically validated at a basic level. The screener should act as a further validation step as well. This

is particularly important for patients with rare diseases, as the incentive often needs to be attractive enough to maximize the sample size.

### Summary

There are several challenges for electronic recruitment and validation of patients with rare diseases for outcomes research studies; however, several measures can be taken to improve study design for the rare disease population. Careful selection of electronic recruitment sources and techniques, a well-designed screener tailored to the study population, comprehensive checks of study data, and if possible, a confirmation of diagnosis by a physician can all increase validation and help achieve accurate, reliable data. •

### Additional information

The preceding article is based on a workshop given at ISPOR Europe 2018. To view the presentation, go to [https://www.ispor.org/docs/default-source/presentations/90361pdf.pdf?sfvrsn=a49f7501\\_0](https://www.ispor.org/docs/default-source/presentations/90361pdf.pdf?sfvrsn=a49f7501_0). For more on ISPOR's Rare Diseases Special Interest Group, go to <https://www.ispor.org/member-groups/special-interest-groups/rare-disease>.

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## The ABCs of Real Option Value of Medical Technologies

Meng Li, ScM, PhD, University of Southern California, Los Angeles, CA, USA, and Louis P. Garrison, PhD, University of Washington, Seattle, WA, USA

**Estimating real option value requires estimating and incorporating future survival and quality of life improvements from adopting new medical technologies, better use of existing technologies, and other changes that can affect survival or quality of life.**

In 2018, the International Society for Pharmacoeconomics and Outcomes Research published a series of Special Task Force reports on US value assessment frameworks, with the goal of informing the shift towards a more value-based healthcare system in the United States. In the reports, several potential novel elements of value—beyond conventional quality-adjusted life-years (QALY) gained and net costs—were identified and defined: insurance value, reduction in uncertainty, fear of contagion, insurance value, severity of disease, value of hope, real option value, equity, and scientific spillovers.<sup>1-2</sup> So far, many of these novel elements of value have been omitted in the theoretical and applied health technology assessment (HTA) literature, and the reports called for more research on developing sound methodologies to estimate them.

This article will address 5 key questions related to one of the novel elements of value—real option value (ROV):

1. What is ROV?
2. What is the origin of real option theory?
3. How large is ROV in healthcare?
4. Does ROV really exist?
5. How does ROV affect value assessment of medical technologies?

### 1. What is ROV?

Real option value is generated when a medical technology that extends the life of patients creates opportunities to benefit from future medical advances. Suppose cancer drug A can extend survival for 1 year, and while patients are on drug A, a new drug B is approved and becomes available to patients that can extend survival for another 1 year after the patients fail drug A. The conventionally calculated survival benefit and QALYs gained of A generally do not account for the possible arrival of B. However, longer survival from drug A is not only valuable in itself, but it also opens up opportunities for patients to benefit from the new drug B in the future. The ROV of drug A is therefore primarily the additional survival (and QALY gain) from the new drug B conditional upon patients surviving to its arrival. Besides ROV from disease-specific technology advancement, there is also ROV from general background

improvements in mortality (ie, for reductions in other causes of death).

### 2. What is the origin of real option theory?

Originating from corporate finance, real options theory recognizes that managers have managerial operating flexibilities—rights, with no obligations to take certain course of action in the future—when operating in a market full of changes, uncertainty, and interrelated decisions.<sup>3</sup> These rights, which are called real options, include deferring, expanding, contracting, abandoning, or altering a project in other ways after it is initiated, as more information about market conditions becomes available. In some cases, especially in infrastructure-based or strategic industries, initial investments (eg, a lease on undeveloped oil reserves) may create subsequent investment opportunities (production and commercializing of oil).<sup>4</sup> These managerial operating flexibilities, or real options, can affect a project's value because management can revise the initial operating plans based on new market development and move its cash flow distribution toward a higher rate of return. By analogy, with real options, payment is made now for the option to make further investments in the future; investing in the current life-extending medical technology can be interpreted as buying an option to benefit from medical advances that are coming through the pipeline.

### 3. How large is ROV in healthcare?

In the current HTA literature, ROV has been measured prospectively in several studies as an increase in expected survival or QALY gain for several drugs in oncology—tyrosine kinase inhibitors, nivolumab, and ipilimumab—in several cancers including chronic myelogenous leukemia, renal cell carcinoma (RCC), squamous non-small cell lung cancer, nonsquamous non-small cell lung cancer, and metastatic melanoma.<sup>5-7</sup> The estimated ROV, measured as additional survival or QALY gain, ranged from 5% of the conventional value for nivolumab for squamous non-small cell lung cancer to 18% for renal cell carcinoma. The size of ROV depends primarily on 2 factors: (1) the survival benefit of the current

life-extending therapy, and (2) the speed of medical technology advancement in the disease area. The greater the survival benefit of the current treatment, the greater the ROV. The faster the technological progress, the greater the ROV. Additionally, many cancer drugs have multiple indications and the ROVs from different indications are potentially additive.

#### 4. Does ROV really exist?

Real option theory implies that a forward-looking patient (optimally informed by a physician agent) would consider both existing treatments and those that are in the pipeline in their current treatment decision making; the future treatment opportunities are the real options here. As a result, their current treatment decisions may change as their expectations about future treatment

**Real option value is generated when a medical technology that extends the life of patient creates opportunities to benefit from future medical advances.**

opportunities change. For example, if a phase II clinical trial demonstrates that an investigational new drug can significantly prolong survival for patients with lung cancer, a rational, well-advised, forward-looking patient may undergo more active treatments so that he/she can live long enough to benefit from the new lung cancer drug. A recent analysis of real-world treatment decisions by melanoma patients with regional or distant metastasis showed that the public disclosure of ipilimumab's phase II result was associated with a nearly twofold immediate increase in the probability of receiving surgical resection of metastasis relative to no treatment.<sup>8</sup> Surgical resection was shown to improve overall survival for metastatic melanoma patients, and this prolonged survival combined with technology advancement (the arrival of ipilimumab) creates ROV. By contrast, the utilization of systemic therapy, which was shown to have no impact on overall survival, did not change significantly in this patient population.

#### 5. How does ROV affect value assessment of medical technologies?

In HTA relying on conventional cost-

effectiveness analysis (CEA), current practice is to evaluate the effect of a treatment in a world where medical technology is fixed and patients passively commit to the treatment assigned, thus neglecting the effect of today's treatment on future treatment opportunities. With the rapid advancement of medical technology and the adoption of a lifetime horizon by many CEAs, these assumptions can be clearly unrealistic in some disease areas. Accounting for ROV in HTA will likely increase the projected QALYs gained of life-extending therapies, as technology is improving and mortality from nearly all causes has been declining in recent decades. Current estimates indicate that the percentage increase ranged from 5% to 18% for a single indication for recent targeted cancer therapies. As a result, a life-extending therapy would be seen as more valuable

by a rational, well-informed plan member than a therapy that provides the same (conventionally calculated) QALYs gained but primarily improves the quality of life. (Improved quality of life may, in theory, generate some ROV as well, as frailty

may limit what treatment a patient can use and its effectiveness.) A life-extending intervention in a disease area with a stronger pipeline and, therefore, with a brighter future would also be seen as more valuable by such a plan member. In addition to the implications for health gains, accounting for ROV in HTA may also be cost-increasing, as future technologies tend to be more expensive than the current ones, due in part to the system-wide rising cost of producing new molecular entities.<sup>9</sup> In the case of ipilimumab for metastatic melanoma, consideration of ROV resulted in approximately a 3% to 7% increase in the incremental cost of ipilimumab.<sup>5</sup> The change in the cost-effectiveness of the therapy, as measured by the incremental cost-effectiveness ratio (ICER), depends on the relative increase in QALYs gained versus the change in cost. In the case of ipilimumab for the treatment of metastatic melanoma, accounting for ROV decreased the ICER by less than 1%.<sup>5</sup>

Estimating ROV requires estimating and incorporating future survival and quality-of-life improvements from adopting new medical technologies, better use of existing technologies, and other changes

that can affect survival or quality of life. Existing studies have used pipeline data projection and mortality data projection for several cancers and have generated relatively consistent findings. As the ISPOR Special Task Force recommended, next steps are to expand the evidence base to other disease areas and to incorporate any trends in quality-of-life improvement over time. Furthermore, work is needed to better understand any interactions among related novel elements of value—especially ROV with insurance value, the value of hope, severity of disease, and scientific spillovers. •

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#### Additional information

The preceding article was based on an issue panel presented at ISPOR 2019. Presentations from this meeting can be found at [www.ispor.org/conferences](http://www.ispor.org/conferences).

# Treatment-Line Versus Patient-Level Matching: A Case Study in Oncology

Xavier Pouwels, MSc, Bram Ramaekers, PhD; Manuela Joore, PhD, Maastricht University Medical Centre+, Maastricht, The Netherlands

**There is an alternative way of performing matching when using data collected from daily clinical practice. Treatment-line matching influences model inputs and results, which ultimately may affect reimbursement decisions.**

### Observational data, confounding by indication, and matching methods

Observational data are increasingly used to inform economic evaluations informing healthcare decision making. However, the biggest threat when using observational data to compare 2 (or more) treatments is the lack of randomization (ie, the comparison is subject to selection bias due to confounding by indication). This means that patients in the treatment groups may have different baseline characteristics that may be related to treatment assignment and the outcome of interest. In those cases, naïvely comparing treatment groups will most likely result in a biased estimate of the treatment's cost-effectiveness. Statistical methods, including regression-based adjustments, matching, and instrumental variables methods, have been developed to address this issue.<sup>1</sup>

Matching methods (eg, propensity score matching and genetic matching) are the main subject of the current article. These methods aim at increasing the similarity in observed baseline characteristics between patients in the intervention and comparator groups. When using propensity score matching, the probability of being assigned to treatment is estimated per patient based on observed baseline characteristics (potential confounders). This probability is then used to match comparator patients with

the most similar baseline characteristics to patients in the intervention group.<sup>2</sup> Genetic matching is a search algorithm that automatically maximizes the similarity in prespecified baseline characteristics between the intervention and comparator groups.<sup>3</sup>

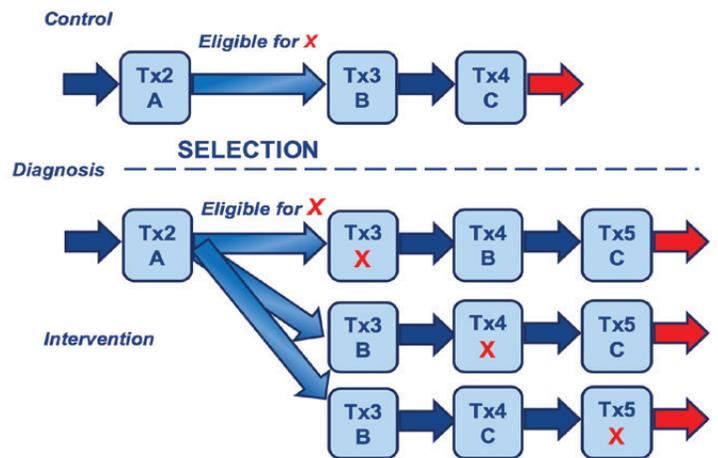
### Why treatment-line matching?

In multiple disease areas such as oncology, rheumatoid arthritis, and cardiovascular disorders, patients typically receive multiple treatment lines, which may create 2 issues. First, comparator patients are usually included in the comparator group at the moment they become eligible for the intervention. This creates an imbalance between the comparator and the intervention group, if (a proportion of) the patients in the intervention group received the intervention later in the treatment pathway than at the moment they became eligible for it. In this situation, patients in the intervention group may be more heavily pretreated than patients included in the comparator group. This imbalance in pretreatment may consequently influence the cost-effectiveness of the intervention versus the comparator. This is illustrated in Figure 1.

Second, the performance of matching methods is influenced by the overlap in baseline characteristics between patients

**Figure 1. Imbalance in pretreatment between control and intervention groups.**

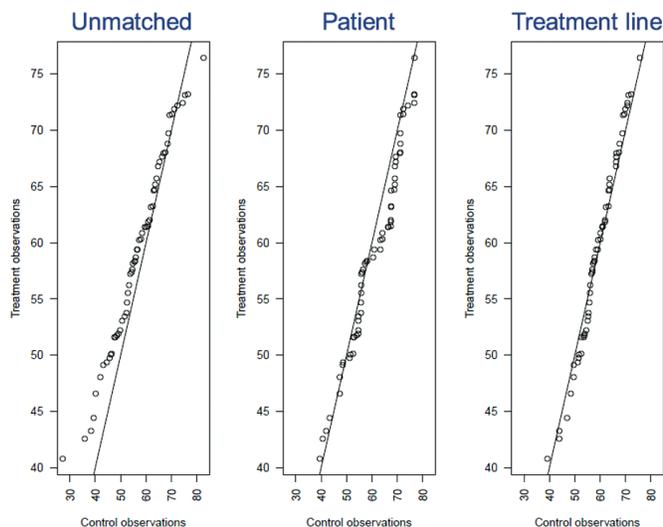
*This illustrates the imbalance in pretreatment between patients who are identified based on the eligibility criteria for the intervention (ie, control), and patients who may receive the intervention later in the treatment pathway (ie, intervention).*



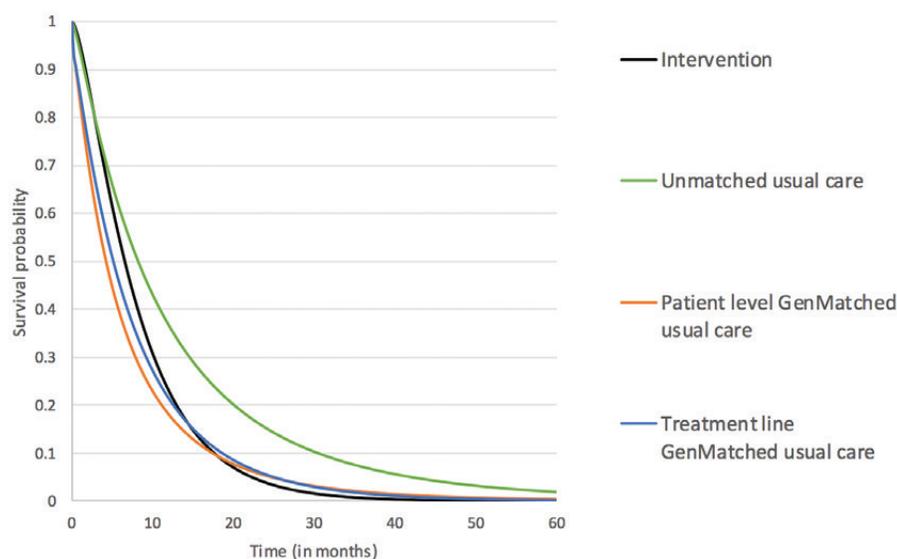
Tx indicates treatment.

**Figure 2. Distribution of baseline age based on the genetic matched populations.**

The illustration represents the distribution of baseline age in the intervention group (y-axis) versus the usual care group (x-axis). Ideally, all dots should lie on the line, which would indicate a perfect overlap of baseline characteristics between the groups.



**Figure 3: Overall survival curves used as model inputs for each group (based on a generalized gamma distribution).**



*GenMatched indicates matched using genetic matching.*

in the intervention and comparator groups and the size of the comparator group. In case of poor overlap in baseline characteristics and small number of comparator patients, matching methods may not be able to increase the similarity in baseline characteristics adequately between the intervention and comparator groups. Additionally, the variance surrounding baseline characteristics in the comparator group may be underestimated.

By considering all treatment lines administered to comparator patients as an individual comparator, the number of

potential comparators is increased and the fact that patients do not receive the intervention when they become eligible for it is reflected in the comparator group. In other words, including treatment lines in the pool of potential comparators results in including different “versions” of the comparator patients in the pool of comparator. This process may be related to matching with replacement, where comparator patients may be included multiple times in the comparator group.

### **An illustration in oncology**

The current case concerns an economic

evaluation of an oncology treatment (the intervention) versus a comparator (usual care). This analysis was based on data collected in daily clinical practice, and the comparison is consequently subject to confounding by indication. Hence, we decided to apply genetic matching to obtain a usual care group that was similar to the intervention group. Since patients in the intervention group had often received the intervention later in the treatment pathway than when they became eligible for it, treatment-line matching might be indicated. We decided to apply both patient-level matching and treatment-line matching to investigate whether treatment-line matching would indeed increase the similarity in baseline characteristics and what the influence of treatment-line matching would be on the results. For completeness, a comparison with the unmatched usual care group was performed. This analysis therefore contains 3 comparisons: (1) intervention versus unmatched usual care, (2) intervention versus patient-level-matched usual care, and (3) intervention versus treatment-line-matched usual care.

The cost-effectiveness model was a 3 health states (progression-free, progressed disease, and death), partitioned survival model. Patients entered the model in the progression-free health state and could either progress or die. Patients in the progressed-disease health state could not transition to the progression-free health state. Effectiveness and resource use estimates were obtained from the database, while utilities and prices were obtained from the literature. In the cost-effectiveness model, progression-free survival and overall survival were estimated through parametric time-to-event models.

In total, there were 90 patients who received the intervention and 321 patients who composed the unmatched usual care group. The 2 matched usual care groups were composed of 90 patients (or treatment lines) each. When analyzing the similarity in baseline characteristics, based on visual inspection of eQQ plots (Figure 2) and a statistical criterion (the bootstrapped Kolmogorev-Smirnov test), we observed that the treatment-line-matched groups were, in general, more similar to the intervention group. >

Treatment-line matching influenced the effectiveness of usual care. The treatment-line usual care group had the longest overall survival estimates than the patient-level-matched usual care group. The unmatched usual care group had the longest survival compared to the matched usual care groups (Figure 3). The unmatched usual care group also had the highest costs associated with the progression-free and progressed-disease health states. The treatment-line-matched usual care group had the longest progression-disease health state

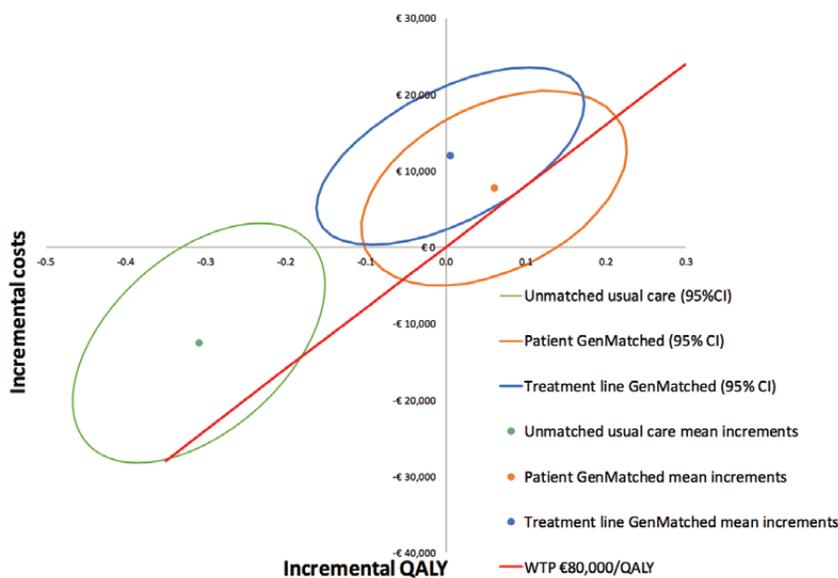
costs than the patient-level-matched usual care group.

These differences in survival and health state costs resulted in differences in total quality-adjusted life years (QALY) gain and total costs obtained by the different usual care groups. The cost-effectiveness of the intervention versus the usual groups was thus affected by whether matching was performed on patients or treatment lines. The intervention was dominated by usual care when compared to the unmatched usual care

group, but was more effective and more costly than the treatment-line-matched and patient-level-matched usual care groups (Figure 4).

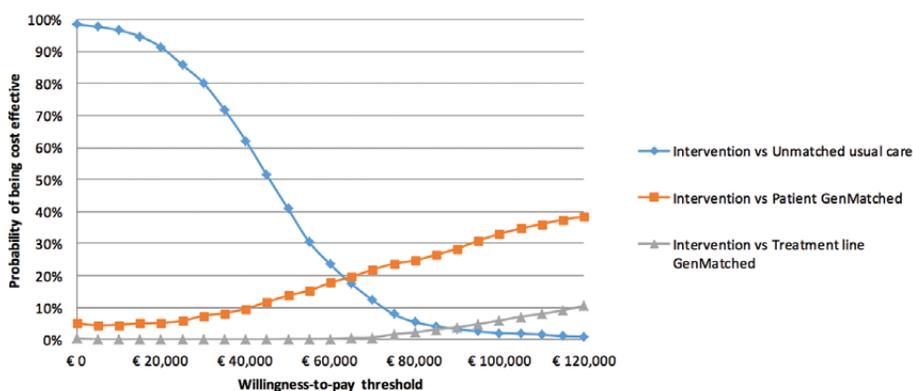
The uncertainty surrounding the results of the comparison of the intervention versus the treatment-line-matched usual care group was lower than the uncertainty surrounding the results of the comparison of the intervention versus the patient-level-matched usual care group. Finally, this resulted in different probabilities of the intervention being cost-effective in each comparison with the usual care groups (Figure 5).

**Figure 4: Incremental costs and QALY for each comparison.**



CI indicates confidence interval; GenMatched, matched using genetic matching; QALY, quality-adjusted life year; WTP, willingness to pay.

**Figure 5: Cost-effectiveness acceptability curve of each comparison.**



CI indicates confidence interval; GenMatched, matched using genetic matching.

## Conclusions

Through this short article, we hope to raise the awareness concerning the possibility of using matching methods on treatment-lines. This case study demonstrates that treatment-line matching improved the similarity in baseline characteristics between the intervention and usual care groups compared to patient-level matching. Treatment-line matching also influenced the model inputs, results, and the uncertainty surrounding the results, which may affect reimbursement decisions.

*NOTE: The empirical data in this article has been systematically modified.*

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# Q&A



## Interview With David Thompson, PhD Outgoing Editor-in-Chief, *Value & Outcomes Spotlight*

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“We are constantly looking to receive high-quality articles written in an accessible manner consistent with how we’ve positioned the publication as a home for non-peer-reviewed contributions.”

As a member of *Value & Outcomes Spotlight*'s Editorial Advisory Board, I've had the distinct pleasure of working with David Thompson, PhD (Syneos Health) for the past few years. As many of you know, Dave has served 3 consecutive terms as editor-in-chief of ISPOR's member publication, and his official tenure in this role will end in June 2020.

As section editor of *Spotlight*'s Q&A column, I wanted to take this opportunity to interview Dave about his journey in this role. In the interview on the following pages, we revisit stories of the publication's evolution and growth, examine some unanswered questions facing the health economics and outcomes research (HEOR) field, and solicit some advice for candidates who may want to take up this post for the next phase in *Value & Outcomes Spotlight*'s development—not only as a publication for ISPOR members, but for the broader HEOR community as well. >

**Santos:** As editor-in-chief, you have led the publication through its transition from a newsletter to a bimonthly HEOR news magazine. Can you describe how the publication has evolved over the years and how the magazine continues to serve the global audience of HEOR readers?

**Thompson:** Twelve years ago, when I first took over as editor-in-chief (originally as co-editor with Thomas Mittendorf) of what was then called *ISPOR Connections*, we immediately recognized that the publication was in the midst of an identity crisis, struggling to find its place alongside ISPOR's flagship peer-reviewed journal, *Value in Health*. Advice was coming from all directions on how best to fix it and there were strong suggestions to make it a peer-reviewed journal focused on health policy. We didn't think ISPOR needed a second peer-reviewed journal, but we knew it needed more than just a simple newsletter—so moving away from the look and feel of a “journal” to that of a “magazine” seemed to make sense. It took a while to get there, but *Value & Outcomes Spotlight* was launched about 6 years later in magazine format.

The second upfront challenge was improving the content quality. When we assessed the article backlog, it seemed to contain a lot of studies that likely had been submitted to and rejected by one or more peer-reviewed journals. So, we put in a lot of work to not only change the format and readability of contributed articles but also their quality. The final phase involved becoming more proactive and self-sufficient in terms of content generation. For the past few years, the *Spotlight* associate editors, editorial advisory board, and I have plotted out in advance the content themes for the 6 issues that comprise each volume. ISPOR has also provided resources to engage a professional writer to draft each issue's theme article, and we are able to solicit additional content externally consistent with the theme as part of the planning process. These changes have made *Spotlight* a more accessible vehicle for communicating HEOR news and information to a wider audience.

**Santos:** In your “Letter from the Editor” that opens every issue, you typically either stir up controversy or bring a

personal connection to the theme—or both. Which of the themes or letters have been the most memorable or impactful for you?

**Thompson:** The opening letter has evolved over time, particularly as the content of each issue of *Spotlight* has become more proactively organized around specific themes. Nowadays, I do my best to introduce the theme, provide my take on it, suggest why it matters to our Society and the readership, and then go on to highlight some of the specific content related to it. This has been the formula for the past year or two. Previously, it was like starting with a blank slate as there was no preplanned theme to focus on, so I would typically look to current events, mostly in the health sector, and provide a blog-style commentary on things. If I had to pick a favorite it would probably be the one I wrote following the death of one of my boyhood idols, Muhammad Ali, in which I highlighted his tremendous contributions as the “voice of the patient” in Parkinson's disease. It meant a lot to me that ISPOR CEO Nancy Berg sent a personal note saying how much she appreciated that one.

**Santos:** *Value & Outcomes Spotlight* recently won the 2019 APEX Award for Publication Excellence. What is the significance of the award to you, to the readers, and to ISPOR?

**Thompson:** This award was a huge surprise, as I didn't even know we were up for consideration. From my perspective, it's a nice pat on the back for all involved and validation of our vision for how *Spotlight* can best serve ISPOR and the broader HEOR community.

**Santos:** While providing the editorial direction for *Value & Outcomes Spotlight* for so many years, what developments or trends have had the most impact in the field of health economics and outcomes research? What are the top 3 challenges or unanswered questions that researchers need to address in the next 5 years?

**Thompson:** A couple things come to mind. Reflecting on recent history, I'm continually amused by the never-ending debate about the QALY [quality-adjusted

life-year]. The measure always seems to have more detractors than supporters, but somehow still manages to beat back all challengers. We've covered this debate and published articles on the impending demise of the QALY, but it's still here—you have to be impressed by its staying power!

Looking forward, I'm intrigued to see how a few ongoing trends play out in the coming years. First, the proliferation of real-world data sources, particularly electronic health records and connected devices, like smart phones and wearables. Second, computing advances for data storage, sharing, linkage, and analysis, especially artificial intelligence, which foreshadows the potential for humans to become bystanders in the process of compiling and analyzing health data. And third, the growing power of the patient—with the eventuality that patients will have control of their own health records and ability to grant access at the push of a button. Think about the possibilities. A payer's computer system independently identifies a problem in care delivery, decides to investigate further, triggers outreach to all relevant patients in the network, asks them to allow access to their historical health records and consent for prospective data collection, collects and analyzes the data, interprets the results, and implements a new care paradigm—all by itself. It's the stuff of science fiction!

**Santos:** How can ISPOR members contribute to *Value & Outcomes Spotlight*?

**Thompson:** The most obvious way is by submitting articles. We are constantly looking to receive high-quality articles written in an accessible manner consistent with how we've positioned the publication as a home for non-peer-reviewed contributions. It's also possible to become part of the editorial advisory board, as current board members typically cycle off after a 4-year term. As things open up, we put out a call for new EAB members in the ISPOR eBulletin—so look for that soon!

**Santos:** As your illustrious term as editor-in-chief comes to a close, what would you say you're most proud of or found most rewarding about this role?

And what would be the best piece of advice you would offer to the incoming editor who takes up this post?

**Thompson:** Overseeing the transformation of *ISPOR Connections* into *Value & Outcomes Spotlight* has been very rewarding. I remember having many conversations about the limitations of *Connections* with ISPOR's Founding Executive Director, Marilyn Dix Smith, and she was initially resistant to making a radical overhaul. I was bothered that the look and feel of *Connections* made it appear like *Value in Health's* underachieving little brother and I knew we would never get out of its shadow without taking things in a completely new direction. So, I kept developing these prototypes of magazine covers for *Connections* that made it look totally different from *Value in Health*. Finally, with the help of Sue Capon and others in ISPOR administration, we had a meeting with Marilyn and she agreed to the change. Once the decision was made, she looked at me and said, "Okay Dave, so what do you propose to call it?"

I wasn't ready for that one so I had to think fast. At the time, my professional title had "Value & Outcomes" in it. So, I said, "How about *Value & Outcomes Spotlight*?" She nodded, looked at everyone around the table, and said "I like it!" And that was that. This was near her retirement from ISPOR and she followed up with a nice phone call the next week confirming that the more she thought about it the more she liked the new direction we were taking things. So, looking back on where we started and how far we've come, it does feel good to have led the creation of a new publication for ISPOR, one that will outlast my tenure as editor.

Along those lines, my advice to the incoming editor-in-chief is to seek incremental improvements immediately, but don't be afraid to take the long view and put into place a plan for more radical improvements over time—even if it requires putting *Spotlight* to bed in favor of something that will better meet the future needs of our Society and the broader HEOR community.

**Santos:** Well, Dave, thanks for sharing your thoughts and stories.

**Thompson:** Thank you, Marisa. I also want to thank everyone who's helped out along the way—there are too many of you to mention by name! I'm looking forward to supporting the next editor-in-chief of *Value & Outcomes Spotlight*. •

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