

2019 TOP10 HEOR TRENDS



ISPOR 2019 TOP 10 HEOR TRENDS

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ISPOR 2019

TOP10 HEOR TRENDS



DRUG SPENDING AND PRICING

This subject has expanded beyond the pricing of pharmaceuticals to encompass drug spending and its impact on payers' healthcare budgets.



GOING BEYOND UNIVERSAL HEALTH COVERAGE

Universal healthcare cannot be universal without ensuring that patients do not face undue barriers to accessing healthcare.



REAL-WORLD EVIDENCE

There is increasing interest and potential for converting real-world data into real-world evidence to inform healthcare decision making



AGING POPULATION

Elder care and long-term care will continue to be global healthcare challenges as the number of people in the world aged 60 years or older continues to grow.





PRICE TRANSPARENCY: NOT JUST ABOUT DRUGS

The lack of transparency in the pricing of healthcare services impedes consumers' healthcare decision making.



"BIG DATA" CONTINUE TO MAKE NOISE

The use of "big data" can assist clinicians in making better healthcare decisions for their patients.





VALUE ASSESSMENT FRAMEWORKS

Value assessment frameworks can be an important element in moving towards a more value-based care model.





HEALTHCARE DECISION MAKING IN LOW-INCOME COUNTRIES

The difference between health technology assessment use by high-income and low-income countries is notable.



PERSONALIZED/PRECISION MEDICINE

As researchers continue to determine the roles that genes play in diseases, HEOR will be needed to evaluate the diagnostics and drugs derived from their discoveries.





UNHEALTHY BEHAVIORS

The root causes of many chronic diseases include a host of unhealthy behaviors that lead to a variety of diseases responsible for the majority of all deaths worldwide.



EXECUTIVE SUMMARY

UST AS IN 2018, 2019 IS SHAPING UP TO BE another significant year for health economics and outcomes research (HEOR), as payers and consumers balance the introduction of new, innovative health technologies, increasing healthcare costs, and the impact of healthcare decisions. ISPOR, the leading global professional society for HEOR, is continuing to monitor the trends that will affect decision makers in healthcare marketplaces around the world. For the second year, based on a survey of its members, the Society has identified the top 10 HEOR trends that will shape the field in the near future. Many of these trends were outlined in the 2018 trends report, but these concerns evolve and shift with time. This 2019 Top 10 HEOR Trends outlines these differences, as well as the new trends that have joined the list.

Of course, a number of important topics and trends exist that do not appear on this year's top 10. Notably, the opioid epidemic is a critically important issue that can benefit from HEOR to help inform clinical and policy decisions. In fact, ISPOR's HEOR publication, *Value & Outcomes Spotlight*, will be dedicating its January-February 2019 issue to the opioid crisis, and this is a topic that will warrant significant attention by the field in the future.

ISPOR members will continue to analyze and explore these trends throughout the year in conferences, summits, and working groups convening around the world.

ISPOR's Health Science Policy Council played an integral role in the development of the Top 10 HEOR Trends initiative. The council serves as an advisory body to the Society's Board of Directors and helps guide ISPOR's focus on key research issues in HEOR.

Methodology for this initiative included a comprehensive exploration and collation of potential trends for consideration; careful review and vetting of the topics by the council; in-depth surveys of ISPOR's membership to provide feedback and rankings of the topics; and final review and discussion of the top 10 trends by the council at its November 2018 meeting held at the Society's ISPOR Europe 2018 conference.

2019 Top 10 HEOR Trends

- 1. Drug spending and pricing
- 2. Universal health coverage and access equity
- 3. Real-world evidence
- 4. Aging population
- 5. Price transparency
- 6. Big data
- 7. Value assessment frameworks
- 8. Healthcare decision making in low-income countries
- 9. Personalized/precision medicine
- 10. Unhealthy behaviors



DRUG SPENDING AND PRICING DRUG BUDGETS AND HEALTH SYSTEM AFFORDABILITY

As in 2018, drug pricing ranked as the number 1 HEOR trend for 2019. The subject has expanded beyond the pricing of pharmaceuticals, now encompassing drug spending and its impact on payers' healthcare budgets. New innovative drugs can save lives, yet can—collectively—become a strain on drug budgets. The challenge is how to provide optimal health outcomes for patients without bankrupting individual patients or the healthcare system.

In May 2018, US President Donald J. Trump unveiled his Blueprint to Lower Drug Prices to address "excessively high drug prices, foreign freeloading, and a system rigged to reward list price increases."

In July 2018, *ISPOR responded* to US Health and Human Services (HHS) Secretary Alex Azar's call for comments on *HHS Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs*, addressing issues such as underpricing or cost-shifting; biosimilar development; value-based arrangement and price reporting; indication-based pricing/payments; long-term financing models of innovative therapies; and informing Medicare beneficiaries about cost-sharing and lower-cost alternatives.²

Some countries are using value frameworks, especially cost-effectiveness analysis, to negotiate drug prices (value frameworks is also trend number 7 in 2019); some are using volume controls (eg, Hungary)³ or more innovative payment arrangements to help control overall spending. The question remains as to how experts can best use health economics tools to help assess drug price versus value and promote efficiency in drug spending. In October 2018, ISPOR held its ISPOR Summit 2018, New Approaches to Value Assessment: Towards More Informed Pricing in Healthcare. The discussions from the summit will help shape ISPOR's activities throughout 2019. ISPOR will offer a series of webinars in 2019 based on several sessions of this summit.

"New innovative drugs can save lives, yet can —collectively—become a strain on drug budgets."



GOING BEYOND UNIVERSAL **HEALTH COVERAGE**

HEALTHCARE CANNOT BE UNIVERSAL WITHOUT ACCESS EQUITY

The number 2 trend, universal health coverage, was in the fourth slot in 2018, but in 2019 the subject goes beyond providing coverage to ensuring that patients do not face undue barriers to accessing healthcare. Although universal health coverage (also known as universal healthcare) is defined as healthcare for all, it is not always implemented through single-payer systems.

Most countries have multiple mechanisms to pay for care even if they have a large primary payer (such as the United Kingdom's National Health Service [NHS]), including a private healthcare sector, funded either as part of an employer-funded healthcare scheme or paid directly by the customer.4

In China, health insurance is provided for publicly and financed by local governments, with 3 main types of publicly financed insurance. However, there are significant differences between those plans, and there are no annual caps on out-of-pocket spending, with most of that spending occurring for prescription drugs.5 A more far-ranging strategy that could result in more universal healthcare access is Healthy China 2030, launched in 2016 by China President Xi Jinping.6

In low- and middle-income countries with ostensibly universal healthcare systems, financial restrictions can bar access. One example is India, where in principle, government health services are available to all citizens under the tax-financed public system. In practice,

however, bottlenecks in accessing such services in India's understaffed and overcrowded public hospitals compel households to seek private care, resulting in high out-of-pocket payments.7 To address care inequities, in September 2018, the Indian government launched Ayushman Bharat, which translates to "Long-Life India," a safety net for the country's poorest 500 million citizens.8

In the United States, the implementation of the Affordable Healthcare Act (ACA) in 2010 has increased healthcare coverage, but coverage overall remains fragmented. Even those with health insurance, either through private insurance or supplemented by the ACA, report that plans with high co-pays and deductibles may make them reluctant to access care.

The World Health Organization has played a critical role in advancing universal healthcare. ISPOR sees universal healthcare as a significant area of interest and is seeking to advance healthcare decisions in this arena.

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3

REAL-WORLD EVIDENCE ITS GROWTH AND POTENTIAL IN REGULATORY DECISION MAKING

Regulatory decision makers continue to evaluate how to use real-world data (RWD) and real-world evidence (RWE) successfully. Technology and integrated electronic medical records have made RWD increasingly accessible and useful for outcomes research and regulatory purposes. Although clinical trial evidence remains the "gold standard" for evaluating treatment efficacy, there is increasing interest and potential for converting RWD into RWE that, through careful analysis and interpretation, can be used to inform healthcare decision making, including regulatory decisions.

"Although RWE has great potential, progress has been slow." RWE offers many benefits over randomized controlled trials, including the availability of timely data at reasonable cost, large sample sizes that enable analysis of subpopulations and less common effects, and the representativeness of real-world practice and behaviors.

While RWE offers tremendous potential, there are concerns about credibility, such as biases due to lack of randomization, data quality, and the potential for spurious results due to data mining.⁹

Although RWE has great potential, when it comes to using it to evaluate new drugs and devices, progress has been slow. The United States in 2016 passed the 21st Century Cures Act, which places additional focus on the use of these types of data to support regulatory decision making. In 2017, the US Food and Drug Administration (FDA) issued a draft guidance, "Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices." The objective of the guidance is to clarify how the agency evaluates RWD to determine if they can be used to generate the type of RWE that the FDA needs to make decisions about medical devices. ¹⁰ In December 2018, the FDA released its *Framework for FDA's Real-World Evidence Program*,

which created "a framework for evaluating the potential use of real-world evidence to help support the approval of a new indication for a drug already approved... or to help support or satisfy drug post-approval study requirements," for public comment.

In Europe, *GetReal* was launched in October 2013, supported by the Innovative Medicines Initiative, a European Union public-private consortium consisting of pharmaceutical companies, academia, health technology assessment (HTA) agencies and regulators (eg, NICE, HAS, EMA, and ZIN), patient organizations, and subject matter experts. The aim of GetReal is to show how robust new methods of RWE collection and synthesis could be adopted earlier in pharmaceutical research and development and the healthcare decision-making process.

ISPOR is dedicated to promoting the use of RWD and RWE through its *Real-World Evidence Strategic Initiative* (ispor.org/RWE). In 2018, ISPOR members collaborated with Duke University's Margolis Center for Health Policy's Real-World Evidence Collaborative to provide input for the FDA's recently published Framework, among other things. This response was published in December 2018.



AGING POPULATION CREATIVE SOLUTIONS NEEDED FOR GLOBAL CHALLENGES

"Many countries are facing a care crisis in that they do not have enough healthcare workers to cover eldercare needs."

Expect elder care and long-term care to be ongoing global healthcare challenges.

This trend, which was number 5 in 2018, advanced to number 4 for 2019. According to the United Nations, between 2015 and 2030, the number of people in the world aged 60 years or older is projected to grow by 56%.¹¹ In the United States, the number of Americans ages 65 and older is projected to more than double by 2060, and the 65-and-older age group's share of the total population will rise to nearly 24%.12

Aging populations mean increases in conditions that require more care, as well as increased healthcare costs. Various countries are trying to address this trend by promoting healthy aging. In Croatia, the Guide for Active Healthy Aging sets out primary, secondary, and tertiary prevention measures.13

While early detection of healthcare problems can help reduce care costs, many countries are facing a care crisis in that they do not have enough healthcare workers to cover eldercare needs. One example of this is Japan, where the country will have nursing care workers to fulfill only 86% of demand in 2025. Helping to depress the numbers of care workers are low wages and high job turnover. Preventive care is also being put forward as part of the solution.14

Mobile health (mHealth), including telemedicine, wearables, and apps, also shows promise in addressing the coming eldercare crisis by expanding services without expanding the existing workforce. Some experts estimate that by 2020, 69% of those over 60 years old will own a mobile device. There are technical challenges to mHealth but it also offers life-changing uses for the elderly population.15



PRICE TRANSPARENCY: NOT JUST ABOUT DRUGS

SUPPORTING CONSUMERS' HEALTHCARE DECISION MAKING

Just as consumers and payers have expressed concerns about the escalating costs of pharmaceuticals, there have been equally vocal comments about the lack of transparency in the pricing of healthcare services. The prices of diagnostics, testing, hospitalizations, surgeries, and elective procedures vary from country to country, and even from hospital to hospital within the same country.

"Transparency in drug pricing has been an issue internationally. due to confidential discounts that are sometimes negotiated."

In the United States, the industry trade group Pharmaceuticals Researchers and Manufacturers of America (PhRMA) found in a survey of consumers that a greater proportion expressed interest in the cost of all healthcare services, not just drugs.16

US President Donald Trump's administration has focused on pharmaceutical pricing, with a significant focus on transparency and rebates. HHS Secretary Alex Azar in October announced a proposal requiring that American drug companies include in their direct-toconsumer (DTC) advertising the list price of any drug paid for by Medicare or Medicaid.¹⁷ In order to share HEOR insights into these situations, ISPOR responded to calls for comments contained in both the original Trump Blueprint and in the DTC proposal.

Transparency in drug pricing has been an issue internationally as well, due to confidential discounts that are sometimes negotiated. Recently, the United Kingdom demanded that a pharmaceutical

company reveal its net prices in other countries for the cystic fibrosis drug Orkambi®. However, a report by the Organization for Economic Co-operation and Development, Pharmaceutical Innovation and Access to *Medicines*, suggested that countries should increase price transparency in pharmaceutical markets but not reveal final agreed prices.

Looking beyond pharmaceuticals, in April 2018, the US Centers for Medicare and Medicaid Services proposed changes to require hospitals to post online a list of their standard charges, as of January 1, 2019.18 With the lack of transparency of medical costs in the United States, some residents have looked into "medical tourism" to find places where they can better afford procedures. One website, medicaltourism.com, offers cost comparisons between the United States and 13 other countries (including Costa Rica, Colombia, India, Mexico, Singapore, and Turkey) on 21 procedures, including cardiac bypass, hysterectomy, knee replacement, and spinal fusion.19

"BIG DATA" CONTINUE TO MAKE NOISE DECISION SUPPORT FOR CLINICAL DECISION MAKING

Just as consumer goods companies use data gathered from their customers to shape marketing campaigns, the use of "big data" can assist clinicians in making better healthcare decisions for their patients.

Helping to shape the use of big data in healthcare are artificial intelligence (AI)/deep learning algorithms. Although the most well-known AI in the healthcare space is IBM Watson, there are others, such as Vyasa, that are creating AI solutions for a wide range of quantitative and qualitative healthcare-related data questions and addressing some key healthcare issues.20

Several healthcare systems have been using big data solutions to improve patient care. Kaiser Permanente's neonatal intensive care units have been using an innovative online calculator to determine whether preterm and newborn babies are at risk of sepsis. Kaiser's clinicians, along with its Division of Research scientists, developed an algorithm for the calculator based on years of information on the vital signs of mothers and babies who acquired sepsis.21

In 2015, Geisinger Health System implemented an IT system called a Unified Data Architecture (UDA) that allowed the company to integrate big data into its

existing data analytics and management systems. The UDA's big data capabilities are used to track and analyze patient outcomes, to correlate their genomic sequences with clinical care, and to visualize healthcare data across cohorts of patients and networks of providers.²²

Even with all of its promise, the adoption of big data in healthcare has lagged behind other industries due to challenges such as privacy of health information, security, siloed data, and budget constraints. But 2 trends will continue to push big data in healthcare: (1) the move from a pay-for-service model, which financially rewards caregivers for performing procedures, to a value-based care model, which rewards them based on the health of their patient populations; (2) and the use of big data analysis to deliver information that is evidence-based and will, over time, increase efficiencies and help sharpen our understanding of the best practices associated with any disease, injury, or illness.23

"Even with all of its promise, the adoption of big data in healthcare has lagged behind other industries."



VALUE ASSESSMENT FRAMEWORKS VALUE-DRIVEN HEALTHCARE AND SHARED DECISION MAKING

Value assessment frameworks can be an important element in moving towards a more value-based care model. A number of value assessment frameworks have been put forward recently by a variety of organizations. ISPOR created its Special Task Force on US Value Assessment Frameworks (ispor.org/ valueframeworks) in 2016 to inform the shift towards a value-driven healthcare system by promoting the development and dissemination of high-quality, unbiased value assessment frameworks.²⁴

Early in 2018, ISPOR's Special Task Force concluded that no single value assessment framework can simultaneously reflect multiple decision contexts and the perspectives of the patient, the health plan, or society as a whole. This means it is particularly important that any framework clearly articulates the value construct it represents and the perspective and decision context in which it is to be used, and to be well validated and reliable within that construct and context. For societal and health plan resource allocation decisions, including coverage and reimbursement decisions, the perspective should reflect, at a minimum, those who ultimately pay for care, including enrollees, employees, and taxpayers. Additionally, well-designed patient-level frameworks can help guide shared decision making for treatment choices among the clinically appropriate options that have been approved for coverage so that patients and their providers can consider and weigh factors most relevant to patient preferences and constraints.25

This Special Task Force also indicated that while costeffectiveness analysis (CEA) is a useful starting point for informing payer and policymaker decisions, elements of costs and benefits not normally included in CEA that affect individual well-being (such as severity of illness, equity, and risk protection) may be relevant for some health plan decisions. However, more research is needed on how best to measure and include them in decision making. ISPOR's October 2018 Summit, New Approaches to Value Assessment: Towards More Informed Pricing in Healthcare, discussed recent developments in current value frameworks as well as the latest research in evaluating how cost-effectiveness and other valuation and decision processes can be used. In 2019, ISPOR will be offering a series of webinars based on several sessions of this summit.

"Well-designed patient-level frameworks can help guide shared decision making for treatment choices."



HEALTHCARE DECISION MAKING IN LOW-INCOME COUNTRIES

SUPPORTING EVIDENCE-BASED DECISIONS AND LAYING THE FOUNDATION FOR HTA

Much of the debate about HEOR and healthcare decision making has focused on high-income countries such as the United States, Germany, France, Japan, and the United Kingdom.

"Low-income countries and 85% of middle-income countries surveyed said they used HTA for planning and budgeting."

One of the main tools that healthcare organizations whether government-based or private—use to evaluate medicines and devices is health technology assessment (HTA). But the difference between HTA use by highincome and low-income countries is notable. According to a 2015 survey by the World Health Organization (WHO), high- and upper middle-income countries, especially in Europe and the Americas regions, were most likely to have a formal HTA process. And low- and middle-income countries with HTA processes were less likely to use them in the same way as high-income countries. WHO found that all low-income countries and 85% of middle-income countries surveyed said they used HTA for planning and budgeting, while only 64% of high-income countries reported using HTA for this purpose. High-income countries were much more likely than low-income countries to use HTA for determining reimbursement or to decide what to include in a package of benefits. Middle-income countries used HTA to inform clinical practice guidelines and protocols (85%) more often than high- and low-income countries (46% and 50%).26

The survey also found a possible link between income level and the focus of HTA. For example, low-income countries tended to use HTA for population-level health interventions (85%), but less often for decisions for medicines (62%), medical devices (54%), or surgical interventions (38%). But a higher proportion of highincome countries reported using HTA for medicines (89%), medical devices (83%), or surgical interventions (69%). While most countries reported having a national entity of more than 6 staff members that produced HTA reports for the ministry of health, organizations in highincome countries were better resourced than those in low-income countries.26

As an organization, ISPOR is committed to supporting HEOR advancement and appropriate HTA use in lowincome and middle-income countries. In 2018 alone, the Society committed \$2.9 million USD to mission-critical initiatives. More than half of ISPOR's regional chapters are based in low- and middle-income countries.²⁷ Additional information on ISPOR's commitment can be found on its Advancing HEOR in Low- and Middle-Income Countries webpage (ispor.org/LMIC). Additional information on HTA Resources can be found at ISPOR's Health Technology Assessment Central (HTAcentral.org).



PERSONALIZED/PRECISION MEDICINE LEVERAGING HEOR TO SUPPORT THE PROMISE OF THE HUMAN GENOME

The entire human genome has been sequenced, so as researchers continue to determine the roles genes play in diseases, HEOR will be needed to evaluate the diagnostics and drugs derived from their discoveries.

Already, technologies such as CAR-T have yielded treatment options, such as Kymriah™ and Luxturna™. The challenge for payers is the high price and one-time dosing schedule of these treatments and other derived gene therapies for cancer and rare diseases. Back in 2015, Peter Bach of Memorial Sloan Kettering Cancer Center proposed that the price of all cancer drugs be tied to the benefits that they offer, a principle shared by value assessment frameworks in general.²⁸ Diagnostics, genetic testing, and evidence-based guidelines for treatment are keys to properly implementing these therapies and determining their value.

In the United States, some patients will face barriers to accessing gene therapies due to the nature of the clinical intervention, the characteristics of the US insurance system, and the interaction between the two. Gene therapies might be very risky for payers because high healthcare costs may cause actuarial risk challenges; high-priced, one-time therapies may create payer budget

pressure; and patients switching from one insurer to another over time may lead to disconnects between the incentives and effects on upstream payers, who face the initial decision to cover the therapy and downstream payers who experience the effects of previous coverage decisions.29

ISPOR's Personalized Medicine Special Interest Group has identified 5 key areas in which HEOR best practices could be developed to improve value assessment, reimbursement, and patient access decisions for personalized medicine. These key areas include research prioritization and early value assessment; best practices for clinical evidence development; best practices for health economic assessment; addressing HTA challenges; and new incentive and reimbursement approaches for personalized medicine.30 The use of RWE and big data is expected to supplement HEOR decisions for gene therapies and other personalized medicines.

"The use of RWE and big data is expected to supplement HEOR decisions for gene therapies and other personalized medicines."



UNHEALTHY BEHAVIORS

MITIGATING THE IMPACT OF PATIENT LIFESTYLES/BEHAVIORS ON DISEASE INCIDENCE AND OUTCOMES

The root causes of many chronic diseases include a host of unhealthy behaviors. Poor diet can lead to hypertension, atherosclerosis, heart disease, and type 2 diabetes. Smoking and vaping can contribute to lung and oral cancers. Alcohol abuse can lead to liver disease.

According to the World Health Organization, 60% of related factors to individual health and quality of life are correlated to lifestyle.31 Also known as "noncommunicable diseases (NCDs)," these conditions are collectively responsible for nearly 70% of all deaths worldwide. Nearly three quarters of all NCD deaths, and 82% of the 16 million people who died prematurely or before reaching 70 years of age, are in low- and middleincome countries. The rise of NCDs has been driven primarily by 4 major risk factors: tobacco use, physical inactivity, the harmful use of alcohol, and unhealthy diets.32

In September 2018, heads of state and governments committed to 13 new steps to tackle NCDs at the Third United Nations High-Level Meeting on Noncommunicable Diseases. These measures include creating robust laws and fiscal measures to protect people from tobacco, unhealthy foods, and other harmful products by restricting alcohol advertising, banning smoking, and taxing sugary drinks; public

education and awareness campaigns to promote healthier lifestyles; vaccinating against human papillomavirus to protect against cervical cancer; treating hypertension and diabetes; and halting the rise of childhood obesity, promoting regular physical activity, reducing air pollution, and improving mental health and well-being.33

One of the ways to modify lifestyle problems that can lead to disease is gamification, the use of game design elements in non-game contexts. Gamification in health also has broad appeal and applicability, cost-benefit efficiency, fits with everyday life, and can support wellbeing.34

For techniques such as gamification, as well as behavioral incentives ("nudges"), HEOR and the evaluation of RWE can be useful in revealing what really works and what does not, hopefully leading to more effective approaches to modifying unhealthy behaviors and the burden of chronic diseases.

"The rise of noncommunicable diseases has been driven primarily by 4 major risk factors: tobacco use, physical inactivity, the harmful use of alcohol, and unhealthy diets"

REFERENCES

- 1. President Donald J. Trump's Blueprint To Lower Drug Prices. May 11, 2018. https://www.whitehouse.gov/ briefings-statements/president-donald-j-trumpsblueprint-lower-drug-prices/. Accessed October 29, 2018.
- 2. Letter to the Hon. Alex M. Azar II, Nancy Berg, July 13, 2018. https://www.ispor.org/docs/default-source/ strategic-initiatives/public-response_2018-07-13_hhsblueprint-to-lower-drug-prices-and-reduce-out-of-pocketcosts.pdf?sfvrsn=6eaf7eb5_0. Accessed October 29, 2018.
- 3. Hren R. Impact of the Pharma Economic Act on diffusion of innovation and reduction of costs in the Hungarian prescription drug market (2007-2010). Value in Health Regional Issues. 2013;2:290-299. https:// www.valuehealthregionalissues.com/article/S2212-1099(13)00076-9/fulltext. Accessed October 28, 2018.
- 4. Chang J, Peysakhovich F, Wang W, Zhu J. The UK Health Care System. Columbia University website. http://assets. ce.columbia.edu/pdf/actu/actu-uk.pdf. Accessed October 28, 2018.
- 5. Fang H. The Chinese Health Care System. International Healthcare System Profiles, The Commonwealth Fund. https://international.commonwealthfund.org/countries/ china/. Accessed October 28, 2018.
- 6. Tan X, Liu X, Shao H. Healthy China 2030: A Vision for Health Care. Value in Health Regional Issues. 12C 2017:112-114. https://www.valuehealthregionalissues.com/article/ S2212-1099(17)30026-2/fulltext. Accessed October 28, 2018.
- 7. Gupta I, Bhatia M. The Indian Health Care System. International Healthcare System Profiles, The Commonwealth Fund. https://international. commonwealthfund.org/countries/india/. Accessed October 28, 2018.
- 8. Withnail A. Modicare: India launches world's biggest experiment in universal healthcare. The Independent UK. https://www.independent.co.uk/news/world/asia/indiahealthcare-modicare-narendra-modi-launch-ayushmanbharat-pm-jay-insurance-a8550526.html. Published September 22, 2018. Accessed October 28, 2018.
- 9. Real-World Evidence. ISPOR Strategic Initiatives. https:// www.ispor.org/strategic-initiatives/real-world-evidence. Accessed October 28, 2018.
- 10. United States Food and Drug Administration. Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices. FDA.gov website.

- 11. United Nations Economic and Societal Affairs. World Population Ageing 2015, UN.org website. http://www. un.org/en/development/desa/population/publications/ pdf/ageing/WPA2015_Report.pdf. Accessed October 28,
- 12. George S, Patel M, Stetz L. The Ticking Time Bomb: Ageing Population. Aetna International website. https://news. aetna.com/2017/10/ticking-time-bomb-aging-population/. Published October 2017. Accessed October 28, 2018.
- 13. Spomenka Tomek-Roksandić, Tomasović Mrćela N, Smolej Naranćić, et al. Program of primary, secondary and tertiary prevention for the elderly. Periodocum Biologorum. 2013;115(4):475-484. https://hrcak.srce.hr/file/172140. Accessed October 28, 2018.
- 14. Fill the gap in nursing care workers. Japan Times. https:// www.japantimes.co.jp/opinion/2018/06/26/editorials/fillgap-nursing-care-workers/#.W9ilplJRdR0. Published June 26, 2018. Accessed October 28, 2019.
- 15. Kruse CS, Mileski M, Moreno S. Mobile health solutions for the aging population: A systematic narrative analysis. | Telemed Telecare. 2017;23(4):439-451. http://journals. sagepub.com/doi/10.1177/1357633X16649790. Published June 1, 2016. Accessed October 28, 2018.
- 16. Zirkelbach R. Americans want to know more than just the list price of a medicine—and we're answering their call. The Catalyst. https://catalyst.phrma.org/americans-wantto-know-more-than-just-the-list-price-of-a-medicine-andwere-answering-their-call. Published October 15, 2018. Accessed October 28, 2018.
- 17. Azar II AM. Remarks on drug pricing to the National Academy of Medicine. The National Academy of Medicine https://www.hhs.gov/about/leadership/secretary/ speeches/2018-speeches/remarks-on-drug-pricing-to-thenational-academy-of-medicine.html. Published October 15, 2018. Accessed October 28, 2018.
- 18. Centers for Medicare & Medicaid Services. CMS proposes changes to empower patients and reduce administrative burden. https://www.cms.gov/newsroom/press-releases/ cms-proposes-changes-empower-patients-and-reduceadministrative-burden. Press release published April 24, 2018. Accessed October 29, 2018.
- 19. Medical-Tourism.com: Your Passport to a World of Options. Medicaltourism.com website. https:// medicaltourism.com/Forms/price-comparison.aspx. Accessed October 29, 2018.

- 20. Identify Patterns & Detect Trends in Healthcare Data Sources. Vyasa website. https://vyasa.com/solutions/ healthcare/. Accessed October 29, 2018.
- 21. Byron J. Big data improves care for Kaiser Permanente's smallest members. Kaiser Permanente Division of Research. https://share.kaiserpermanente.org/article/ big-data-improves-care-for-kaiser-permanentes-smallestmembers/. Published June 6, 2014. Accessed October 30, 2018.
- 22. Erskine AR, Karunakaran B, Slotkin JR, Thomas DA. How Geisinger Health System uses big data to save lives. Harvard Business Review. https://hbr.org/2016/12/howgeisinger-health-system-uses-big-data-to-save-lives. Published December 15, 2016. Accessed October 30, 2018.
- 23. Healthcare big data and the promise of value-based care. NEJM Catalyst. https://catalyst.nejm.org/big-datahealthcare/. Published January 1, 2018. Accessed October 30, 2018.
- 24. Value Assessment Frameworks. ISPOR. https://www.ispor. org/strategic-initiatives/value-assessment-frameworks. Accessed October 30, 2018.
- 25. Garrison LP Jr, Neumann PJ, Willke RJ, et al. A health economics approach to US value assessment frameworks—summary and recommendations of the ISPOR Special Task Force Report. Value in Health. 21(2):161-165. https://www.valueinhealthjournal.com/ article/S1098-3015(17)33894-9/fulltext. Accessed October 30, 2018.
- 26. World Health Organization. 2015 Global Survey on Health Technology Assessment by National Authorities. http://www. who.int/health-technology-assessment/MD_HTA_oct2015_ final_web2.pdf?ua=1. Accessed November 1, 2018.
- 27. ISPOR. Advancing HEOR in low and middle income countries. ISPOR website. https://www.ispor.org/about/ our-mission/advancing-HEOR-in-low-and-middle-incomecountries. Accessed November 1, 2018.

- 28. Weintraub A. Here come more gene therapies—and more pricing debates. Forbes. October 11, 2017.
- 29. Cirametaro M, Long G, Johnson M, Kirson N, Dubois RW. Are payers ready to address the financial challenges associated with gene therapy? Health Affairs Blog. https://www.healthaffairs.org/ do/10.1377/hblog20180626.330036/full/?utm. Published June 28, 2018. Accessed November 1, 2018.
- 30. Challenges in the Development and Reimbursement of Personalized Medicine—Payer and Manufacturer Perspectives and Implications for Health Economics and Outcomes Research: A Report of the ISPOR Personalized Medicine Special Interest Group. https://www.ispor.org/ docs/default-source/sig-documents/Challenges-in-the-<u>Development-and-Reimbursement-of-Personalized-</u> Medicine.pdf. Published June 28, 2018. Accessed November 1, 2018.
- 31. Farhud DD. Impact of lifestyle on health. Iran J Public Health. https://www.ncbi.nlm.nih.gov/pmc/articles/ PMC4703222/#B1. Published November 2015. Accessed November 1, 2018.
- 32. World Health Organization. Noncommunicable diseases and their risk factors. http://www.who.int/ncds/en/. Accessed November 1, 2018.
- 33. World Health Organization. Heads of State commit to lead response to beat noncommunicable diseases, promote mental health. http://www.who.int/news-room/ detail/27-09-2018-heads-of-state-commit-to-leadresponse-to-beat-noncommunicable-diseases-promotemental-health. Published September 27, 2018. Accessed November 1, 2018.
- 34. Johnson D, Deterding S, Kuhn KA, Staneva A, Stoyanov S, Hides L. Gamification for health and wellbeing: A systematic review of the literature. Internet Interv. 2016;6:89-106. https://www.sciencedirect.com/science/ article/pii/S2214782916300380. Published November 2016. Accessed November 1, 2018.

DEVELOPMENT OF THE ISPOR 2019 TOP 10 HEOR TRENDS

ISPOR—the professional society for health economics and outcomes research (HEOR)—is an international, multistakeholder scientific and educational nonprofit organization that is recognized globally as the authority in HEOR and its use in decision making to improve health. ISPOR is the leading source for scientific conferences, peer-reviewed and MEDLINE®-indexed publications, good practices guidance, education, collaboration, and tools/resources in the field. As the leading professional society in HEOR, ISPOR is uniquely positioned to provide direction on trends in the field. ISPOR's Health Science Policy Council, in conjunction with the Society's Chief Science Officer Richard J. Willke, PhD and Associate Chief Science Officer Lucinda Orsini, DPM, MPH, has led efforts in developing a list of the 2019 Top 10 HEOR Trends.

ISPOR's Health Science Policy Council

The ISPOR Health Science Policy Council was established as an advisory council to the Board of Directors in 2004 to ensure that the Society is addressing key research issues in outcomes research. Membership in the Health Science Policy Council is composed of invited members, including ISPOR past presidents, Avedis Donabedian Lifetime Achievement Award honorees, and other key thought leaders from the ISPOR membership base. In addition to its involvement in the HEOR trends initiative. the Health Science Policy Council also serves as an advisory body for the Society through horizon-scanning efforts and critical review and oversight of proposals for ISPOR's Good Practices for Outcomes Research Task Forces.

The Health Science Policy Council was reorganized in 2017 to include 3 key committees—the Policy Outlook Committee, the Science Research Committee, and the Task Force Review Committee. These committees are co-chaired by Health Sciences Policy Council members and include representatives from other ISPOR groups, including the Institutional Council, Student Chapter Faculty Advisors Council, Health Technology Assessment Council, Patient Council, Education Council, Latin America Consortium, Asia Consortium, Central and Eastern Europe Consortium, Africa Network, Arabic Network, and the Co-Editors-in Chief of Value in Health, as well as the ISPOR Chief Science Officer.

Methodology

The methodology for development of the 2019 Top 10 **HEOR Trends included:**

Topic Exploration

Comprehensive exploration to generate a "long list" of potential topics was conducted by examining HEORrelated topics at a variety of industry conferences (including ISPOR conferences and other industry events), articles in scientific journals, research/industry blogs, and articles in trade publications.

Review and Vetting

ISPOR's Health Science Policy Council (including its committees) reviewed and vetted the "long list" at its council meeting at the ISPOR 2018 conference held in May 2018 in Baltimore, Maryland, United States, to generate a "short list" of more than 40 potential HEOR trends for consideration.

Thought Leader Survey

A survey of ISPOR members was conducted to rate the topics curated by the Health Science Policy Council.

Finalization

The Health Science Policy Council and its committees reviewed and finalized selections for the Top 10 list based on the survey results from its meeting at the ISPOR Europe 2018 conference held in November 2018 in Barcelona, Spain.

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