

2020 TOP 10 HEOR TRENDS



ISPOR 2020 TOP 10 HEOR TRENDS

CONTENTS

	TOP 10 HEOR TRENDS	2
	EXECUTIVE SUMMARY	3
TREND 1	REAL-WORLD EVIDENCE	4
TREND 2	DRUG PRICING	5
TREND 3	NOVEL CURATIVE THERAPIES	6
TREND 4	OVERALL HEALTHCARE SPENDING	7
TREND 5	UNIVERSAL HEALTH COVERAGE— ACCESS AND EQUITY	8
TREND 6	VALUE-BASED ALTERNATIVE PAYMENT MODELS	9
TREND 7	PRICE TRANSPARENCY	10
TREND 8	DIGITAL TECHNOLOGIES	11
TREND 9	AGING POPULATION	12
TREND 10	PRECISION MEDICINE	13
	REFERENCES	14
	DEVELOPMENT OF THE ISPOR 2020 TOP 10 HEOR TRENDS	17
	ACKNOWLEDGMENTS	18

ISPOR 2020 TOP 10 HEOR TRENDS

- 1 Real-World Evidence**

Real-world evidence in healthcare decision making has risen in this year's trends list due to a number of converging factors.


- 2 Drug Pricing**

Pressure is increasing on drug makers as to how they price their products.


- 3 Novel Curative Therapies**

Many of these medicines represent great strides forward in treatment; however, their pricing may put them out of the reach of many patients.


- 4 Overall Healthcare Spending**

WHO reports that the world spent \$7.5 trillion on health, representing close to 10% of global GDP.


- 5 Universal Health Coverage—Access and Equity**

Universal healthcare will remain an important issue as many countries still seek to provide their citizens with healthcare.


- 6 Value-Based Alternative Payment Models**

Innovative, high-cost therapies drive the search for novel payment models.


- 7 Price Transparency**

Lack of clarity about information on pricing for healthcare products and services impacts healthcare budgets and patients.


- 8 Digital Technologies**

A new topic for 2020, digital healthcare is advancing rapidly with the potential to transform healthcare delivery and outcomes assessment.


- 9 Aging Population**

This global demographic trend will have a long-term impact on healthcare delivery and costs for some time to come.


- 10 Precision Medicine**

Precision, or personalized, medicine is a growing field that intersects with big data.



EXECUTIVE SUMMARY

THE 2020 VISION FOR HEALTH ECONOMICS AND OUTCOMES RESEARCH IS COMING INTO FOCUS AND THE TRENDS IDENTIFIED IN 2018 AND 2019 CONTINUE TO BE SIGNIFICANT FOR PATIENTS, PAYERS, AND HEALTHCARE PROVIDERS.

ISPOR, the leading global professional society for health economics and outcomes research (HEOR), is continuing to monitor the trends that will affect decision makers in healthcare marketplaces around the world. For the third 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. Some of these topics were outlined in the 2018 and 2019 trends reports, but these trends evolve and shift with time. This *2020 Top 10 HEOR Trends* outlines these differences, as well as the new trends that have joined the list.

Rising to the top of the list of trends for 2020 is the impact of real-world evidence. Other topics that stayed on the list from 2019 are drug pricing, universal health coverage, price transparency for healthcare products and services, healthcare for an aging population, and personalized/precision medicine. New to the list this year are affordability for novel, curative therapies (outside of general drug-pricing issues); overall healthcare spending (separated from the general “pricing” issues); value-based alternative payment models for therapies; and the role of digital technologies in healthcare delivery and outcomes assessment.

It is notable that 5 of this year’s 10 trends address the finances of healthcare in some form. Trend number 2 is drug pricing. Trend number 3 is the affordability of novel and curative therapies. Trend number 4 is overall healthcare spending. Trend number 6 is value-based alternative payment models. And trend number 7 is price transparency for healthcare products and services. These issues of healthcare pricing, affordability, spending, and payment models will be with us for some time. HEOR is

uniquely poised to help provide relevant evidence and inform decision making related to the economics of healthcare.

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

The 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.

2020 Top 10 HEOR Trends

1. **Real-World Evidence**
2. **Drug Pricing**
3. **Novel Curative Therapies**
4. **Overall Healthcare Spending**
5. **Universal Health Coverage—Access and Equity**
6. **Value-Based Alternative Payment Models**
7. **Price Transparency**
8. **Digital Technologies**
9. **Aging Population**
10. **Precision Medicine**

1



REAL-WORLD EVIDENCE RWE IN HEALTHCARE DECISION MAKING

In 2019, real-world evidence (RWE) was identified as trend number 3 by ISPOR members. RWE has risen to number 1 in this year's trends list due to a number of converging factors.

Regulatory bodies, such as the US Food and Drug Administration (FDA), the United Kingdom's National Institute for Health and Care Excellence (NICE), and the European Medicines Agency are exploring how to gather and analyze data to increase the supportive use of real-world data (RWD) and RWE in regulatory decision making. Additionally, healthcare providers and payers (government and private) want to determine how to use RWE to establish comparative- and cost-effectiveness. These influences and others have further increased the importance of RWE and its role in informing healthcare decisions worldwide.

The US FDA issued a draft guidance in May 2019, "Submitting Documents Using Real-World Data and Real-World Evidence to FDA for Drugs and Biologics Guidance for Industry." According to the guidance, submissions using RWE and RWD "can be in different forms such as a new protocol(s) submitted to an existing [investigational new drug application], a final study report submitted to a [new drug application] or [biologics license application] supplement, or a meeting package that discusses the use of RWE."¹ In June 2019, NICE proposed extending its use of data for regulatory decisions. The organization was already using a wide range of published scientific evidence for its guidance for the United Kingdom's National Health Service (NHS) on health technologies and its advisory guidelines. NICE is now proposing to extend its use of data from sources including audits of procedures such as operations; registries that collect data on how particular treatments are used; surveys of people using services; and data collected on national trends.²

According to an analysis published in April 2019 by the American Society for Clinical Pharmacology and Therapeutics' journal, "from a European perspective, utilizing RWD is faced with operational, technical, and methodological challenges." Possible solutions include early and repeated consideration of the need for RWD during drug development; long-term funding for data infrastructures; use of common data elements, data formats and terminologies, or mapping to an international system; and detailed description of study design and data collected in data sources.³

ISPOR has continued to focus on RWE and RWD in its special task forces, special interest groups, and strategic initiatives. Through its RWE strategic initiative, ISPOR is actively working to improve standards and practices for the collection and analysis of RWD.⁴ In 2019, ISPOR entered a partnership with the International Society for Pharmacoepidemiology, the Duke-Margolis Center for Health Policy, and the National Pharmaceutical Council to form the RWE Transparency Initiative. The Initiative is focused on establishing a culture of transparency for study analysis and reporting of RWE studies on treatment effects meant for use in healthcare decision-making.⁵ The partnership issued a draft white paper in September 2019, which outlines an approach designed to facilitate the registration of hypothesis evaluating treatment effectiveness (HETE) studies based upon secondary data use such as insurance claims and electronic health records, particularly those testing hypotheses regarding effectiveness and/or safety of 2 or more interventions.⁶

"Through its RWE strategic initiative, ISPOR is actively working to improve standards and practices for the collection and analysis of RWD."

2



DRUG PRICING PRESSURE INCREASES TO CONSTRAIN THE PRICING OF MEDICINES

In the 2019 trends report, the pricing of drugs was discussed as part of the issue of how much of the total healthcare spend was for medicines, but for 2020, the pressure is increasing on drug makers as to how they price their products, especially in the United States.

Foremost in the news cycle was the pricing of insulin. In April 2019, the US House Committee on Energy and Commerce held a day-long hearing, “Priced Out of Lifesaving Drug: Getting Answers on the Rising Cost of Insulin,” where lawmakers questioned representatives of drug makers and pharmacy benefit managers on how they set prices for the drug. This was the second hearing that the House has held on insulin costs.⁷

Although insulin is the “poster child” for drug pricing issues in the United States, the pricing of all drugs has come under attack by critics. In response, the Trump administration proposed the adoption of a reference pricing system in 2018 for Medicare Part B drugs, in which drugs would be indexed to international prices.⁸ In September 2019, US House of Representatives Speaker Nancy Pelosi put forward HR-3, the Lower Drug Costs Now Act, which proposes giving Medicare the power to negotiate directly with drug companies and would provide all Americans with private insurance access to those same negotiated drug prices.⁹

Results from a poll from West Health and Gallup released in 2019, “The US Healthcare Cost Crisis,” found that 58 million Americans reported an inability to pay for needed drugs in the past year.¹⁰ Even in countries with universal

healthcare, such as Canada, drug costs can be a concern. One study published in October 2019 by the University of Illinois showed that food-insecure Canadian households may have trouble affording prescription medication, which can contribute to higher disease rates among this population.¹¹

Despite the debate, not much progress has been made to change the current system as the pricing issue butts up against fears of harming innovation. Speaker Pelosi’s plan has already come under fire from the heads of small biotechs, who fear that if the plan passes, it will dry up the venture capital that is necessary for startups to get off the ground.¹²

During a session at the ISPOR Europe 2019 conference in November, “Time for Change? Has the Time Come for the Pharma Industry to Accept Modest Prices?,” panelists offered contrasting views on how the biopharmaceutical industry can deliver maximum return on investment without compromising patient access. As panelists at this session stressed, balancing the needs of patients, healthcare systems, drug innovators, and investors has never been more difficult; however, failure to do so could have disastrous consequences for all stakeholders.¹³

“Despite the debate, not much progress has been made to change the current system as the pricing issue butts up against fears of harming innovation.”

3



NOVEL CURATIVE THERAPIES AFFORDABILITY IMPACTS ACCESS FOR PATIENTS

2019 was marked by several approvals of novel drugs—21 novel products were recorded by the FDA as of November.

These novel curative therapies include Zolgensma[®], to treat young children with spinal muscular atrophy;¹⁴ Xenleta[™], to treat adults with community-acquired bacterial pneumonia; Trikafta[™], the first triple-combination therapy to treat patients with the most common cystic fibrosis mutation; and Reblozyl[®], for the treatment of anemia in adult patients with beta thalassemia who require regular red blood cell transfusions.¹⁵

While many of these medicines represent great strides forward in treatment for these conditions, their pricing may put them out of the reach of many patients—with payers having to restrict access due to budget constraints. However, many of the conditions these drugs treat involve very small patient populations. For example, Zolgensma is a gene therapy for children less than 2 years old with spinal muscular atrophy (SMA) that targets the genetic root cause of SMA by replacing the function of the missing or nonworking survival motor neuron 1 (SMN1) gene.¹⁶ Fewer than 1000 infants in the United States are born with SMA and Zolgensma costs \$2.125 million for the single required infusion.¹⁷

In the United States, the Institute of Clinical and Economic Review (ICER) has been evaluating the cost/value for cystic fibrosis transmembrane conductance regulator (CFTR) modulators. In its first report on the then-available drugs—Symdeko[®], Orkambi[®], and Kalydeco[®], all by Vertex Pharmaceuticals—ICER's panel found that these drugs

offer a net health benefit compared to best supportive care alone and provide other benefits such as reduced caregiver burden and new options for patients in whom other therapies have not been effective. But the majority of the Midwest Comparative Effectiveness Public Advisory Council voted that the therapies represent a low long-term value for money, due in large part to their high costs. ICER's analysis suggested that discounts of up to 77% would be necessary to bring the prices into alignment with the drugs' clinical value to people with cystic fibrosis and their families.¹⁸ ICER is conducting another review of CFTR modulators in April 2020, this time including Trikafta, at the California Technology Assessment Forum, which will vote on ICER's report.¹⁹

In a September 2019 paper comparing how countries outside of the United States evaluated and valued drugs for rare conditions, ICER concluded that there is no magic solution to assessing the evidence on clinical effectiveness, economic impact, and value of drugs to treat rare diseases. "The way forward will therefore require a coalescing of approaches internationally to define the level of rarity requiring special action, to determine what adaptations of traditional [health technology assessment] HTA are required, the types of special pricing and access considerations that are necessary, and willingness among major US decision makers to fully embrace these approaches."²⁰

"While many of these medicines represent great strides forward in treatment for these conditions, their pricing may put them out of reach of many patients."

4



OVERALL HEALTHCARE SPENDING GROWTH IN SPENDING EXCEEDS THAT OF GDP

“These issues are ones where HEOR can play an especially important role.”

The World Health Organization (WHO), in its report *Public Spending on Health: A Closer Look at Global Trends*, found that in 2016, the world spent \$7.5 trillion on health, representing close to 10% of global gross domestic product (GDP). Additionally, total health spending is growing faster than GDP, increasing more rapidly in low- and middle-income countries (close to 6% on average) than in high-income countries (4%).²¹

But what are we getting for the billions of healthcare dollars spent? In the United States, a lot less than we think. An October 2019 article in *JAMA*, “Waste in the US Health Care System: Estimated Costs and Potential for Savings,” calculated that the total annual costs of waste in 6 “waste domains” (failure of care delivery; failure of care coordination; overtreatment or low-value care; pricing failure; fraud and abuse; and administrative complexity) were \$760 billion to \$935 billion and savings from interventions that addressed waste were \$191 billion to \$282 billion.²²

The 34 countries in the Organisation for Economic Co-operation and Development (OECD) are also struggling with waste in healthcare spending. The OECD reported that in 2017, “A significant share of health spending in OECD countries is at best ineffective and at worst, wasteful.” The OECD suggests that up to one-fifth of health spending could be channeled towards better use. “With over 9% of GDP spent on healthcare systems across the

OECD, three-quarters of which is paid for by governments, such waste undermines the financial sustainability of our health systems,” the report says, and suggests measures to curb waste, such as halting spending on unnecessary surgeries and clinical procedures, encouraging the use of generic drugs, developing advanced roles for nurses for chronic patient management, or ensuring that patients who do not require hospital care are treated in less resource-consuming settings such as primary care.²³

These issues are ones where HEOR can play an especially important role. HEOR can provide comparative evidence that can, among those products and services that drive healthcare spending, identify those that improve patients’ health outcomes, provide the most value, and lead to reduced waste. Such evidence is essential to be able to identify how to best deploy healthcare budgets to benefit patients.

5



UNIVERSAL HEALTH COVERAGE HEALTHCARE COVERAGE, ACCESS, AND EQUITY

In 2019, universal health coverage (also known as universal healthcare) was the number 2 trend. It continues to be a vital issue in healthcare and remains in the top 5 for 2020.

“WHO estimates that at least half of the world’s population still does not have full coverage of essential health services.”

The costs of pharmaceuticals and medical procedures and the lack of medical coverage continue to be strongly debated in the United States. As the 2020 elections approach, the drumbeat is sounding louder for universal healthcare, with Democratic presidential candidates Elizabeth Warren and Bernie Sanders forwarding their own plans for “Medicare for All.” While Warren’s proposal is less sweeping than Sanders’, experts agree that it is more expansive than those offered by Democratic rivals Pete Buttigieg and former Vice President Joe Biden.²⁴

The lack of healthcare coverage for all citizens is not just a problem for the United States. WHO estimates that at least half of the world’s population still does not have full coverage of essential health services, with about 100 million people being pushed into extreme poverty (defined as living on \$1.90 or less a day) due to their own personal healthcare expenditures. Additionally, more than 800 million people (almost 12% of the world’s population) spent at least 10% of their household budgets to pay for healthcare.

WHO defines universal health coverage (UHC) as a system in which “all individuals and communities receive the health services they need without suffering financial hardship. It includes the full spectrum of essential, quality health services, from health promotion to prevention, treatment, rehabilitation, and palliative care.” WHO notes that primary healthcare is the most efficient and cost-

effective way to achieve universal health coverage around the world.

But as WHO points out, universal healthcare does not mean free healthcare and does not entail only health financing, but encompasses all components of the health system: health service delivery systems, the health workforce, health facilities and communications networks, health technologies, information systems, quality assurance mechanisms, and governance and legislation.

All United Nations Member States have agreed to try to achieve UHC by 2030, as part of the Sustainable Development Goals. WHO and the World Bank have developed a framework to track the progress of UHC. This monitoring will focus on (a) the proportion of a population that can access essential quality health services and (b) the proportion of the population that spends a large amount of household income on health.²⁵

Universal healthcare will remain an important issue for some time to come as many countries still seek to provide healthcare to their citizens. WHO continues to play a critical role in advancing universal health coverage. As the issue of universal health coverage evolves, the field of HEOR will help to inform decisions, develop health technology assessment competencies, and make the best possible decisions about how to spend healthcare budgets for optimal health outcomes.



6

VALUE-BASED ALTERNATIVE PAYMENT MODELS DETERMINING THE TRUE VALUE OF INNOVATIVE THERAPIES

What is the true value of a therapy, and how should the price be set? With new therapies being approved that promise greater benefits to patients but come at a correspondingly eye-watering price, both public and private payers are trying to figure out how to pay for treatments without bankrupting the system.

Although health technology assessment (HTA) bodies should be able to distinguish between therapies that provide good value from those that provide poor value, often there is a lack of evidence when the therapy is new. If patients are waiting for a new therapy that promises great benefits, payers will often approve the therapy and reassess when evidence is generated. In the United States, the term “coverage with evidence development” is used, which was created by the Centers for Medicare and Medicaid Services (CMS) in 2005, but the concept existed before in other countries with different names used, according to Urs Brügger, in the 2014 paper, *A Review of Coverage With Evidence Development (CED) in Different Countries: What Works and What Doesn't*.²⁶

For therapies that have already proven their value to patients yet come at a price that creates worries that not all patients who can benefit could be covered, payers are already looking at other methods. One that has drawn headlines is the subscription-based model, often referred to as the “Netflix model.” In the United States, the state of Louisiana innovated this model to pay for hepatitis C therapies, which CMS approved in June 2019. Under this plan, Louisiana negotiated supplemental rebate agreements for Medicaid and for incarcerated patients, capping gross expenditures at a fixed amount while retaining unlimited access to the antiviral hepatitis C treatment.²⁷ The subscription-based payment model is

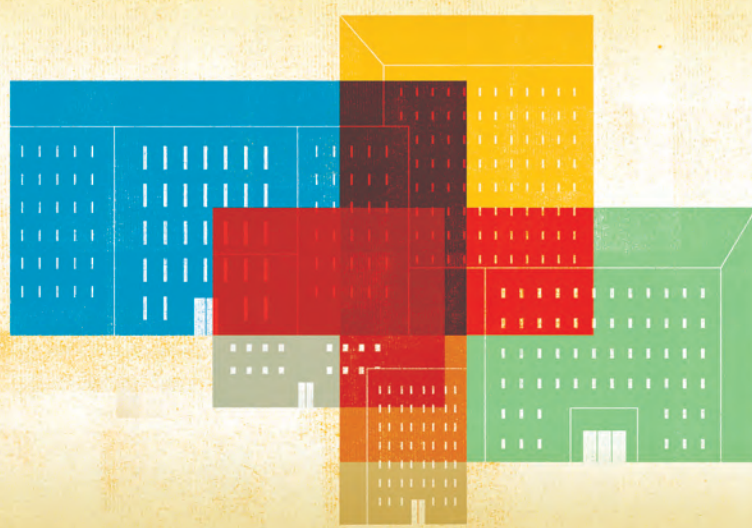
also being used now by Australia and Washington state in the United States for hepatitis C therapies.

For other transformative therapies such as oncology drugs, in 2014, a Massachusetts Institute of Technology economist and Harvard oncologist proposed a “mortgage model,” in which patients can secure “healthcare loans [HCLs].” “HCLs allow patients in both multipayer and single-payer markets to access a broader set of therapeutics, including expensive short-duration treatments that are curative. HCLs also link payment to clinical benefit and should help lower per-patient cost while incentivizing the development of transformative therapies rather than those that offer small incremental advances,” the authors argue in their paper for *Science Translational Medicine*.²⁸

Performance-based risk-sharing is an alternative payment model that links payment to the actual performance, or health outcomes, of the drug or other health intervention. This model is also referred to as outcomes-based or value-based payment. ISPOR's Good Practices for Performance-Based Risk-Sharing Arrangements Task Force has outlined in its 2013 report, *Performance-Based Risk-Sharing Arrangements—Good Practices for Design, Implementation, and Evaluation*, how to develop and apply state-of-the-art methods when considering, using, or reviewing performance-based risk-sharing arrangements.²⁹

“One [payment model] that has drawn headlines is the subscription-based model, often referred to as the “Netflix model.”

7



PRICE TRANSPARENCY FOR HEALTHCARE PRODUCTS AND SERVICES

LACK OF CLARITY ABOUT PRICES IMPACTS HEALTHCARE BUDGETS AND PATIENTS

One of the most fiercely debated areas of healthcare has been cost—as in, what does a drug, device, or procedure actually cost? In the European Union, prices vary by country; in the United States, they vary by healthcare provider.

In the United States, President Trump, in June 2019, issued an executive order, “Improving Price and Quality Transparency in American Healthcare to Put Patients First.” The order directed the Secretary of Health and Human Services to issue a regulation that would require hospitals to publicly post costs of services in an easily understandable format and seek comment on a proposal that would require insurers and providers to give information about out-of-pocket costs before patients receive care.³⁰

Unique to the United States is the intersection of price transparency and drug rebates with pharmacy benefit managers (PBMs). The drug-pricing rebate system has benefited the financial bottom lines of PBMs while raising concerns that rebates contribute to increased drug prices. Calls for increased regulation are placing pressure on PBMs to increase price transparency.

Pro Publica estimates that more than half the debt in collections in the United States stems from medical care. Unlike most other debt, people often receive healthcare without really knowing the true costs or by having opportunities to shop around. In Coffeyville, Kansas, patients and their families can expect to be arrested for medical debt if they miss court hearings to set up a payment for that debt; Pro Publica describes it as “a government-sanctioned shakedown of the uninsured and underinsured.”³¹

The European Union (EU), where each member state sets up its own prices for drugs and healthcare procedures, has examined the prospects of health services competition, and since 2011 has allowed patients to seek cross-border care and be reimbursed by that care.³² But a June 2019 report released by the European Court of Auditors (ECA) found that so far, very few patients ask for reimbursements for treatment accessed. The ECA report noted that only a minority of patients are aware of their right to seek medical care abroad, but additional discouragements are that patients are required to pay for services up front and only get reimbursed for the price at which their home country has priced the treatment.³³

Pricing transparency measures in the European Union have been designed to ensure transparency and to verify that national pricing and reimbursement decisions do not create obstacles to the pharmaceutical trade within the European Union’s Internal Market—based on the 1988 “Transparency Directive” that they are seeking to update.³⁴ After the European Union’s inquiry into the pharmaceutical sector in 2009, the national competition authorities of EU member states have adopted 29 antitrust decisions against pharmaceutical companies, investigating and sanctioning practices that led to higher drug prices.³⁵

“Pro Publica estimates that more than half the debt in collections in the United States stems from medical care.”

8



DIGITAL TECHNOLOGIES DIGITAL IN HEALTHCARE DELIVERY AND OUTCOMES ASSESSMENT

“The large technology giants have entered healthcare with broad visions.”

What once was science fiction has become a reality, as Amazon’s Echo and Google’s Home devices bring us Alexa and Google Assistant that can assist us with shopping lists, find the music we want to listen to, and tell us the news and weather. But digital technology is already starting to play a role in healthcare and that role will only grow.

A new topic for the 2020 trends list, digital technologies are just beginning to upend healthcare as we know it. The impact of digital is far reaching and touches virtually every area of the healthcare system—wearables, digital apps as diagnostics and therapies, medical devices, electronic medical records, telemedicine, and big data, to name just a few.

The large technology giants have entered healthcare with broad visions. Google seeks to apply its artificial intelligence (AI) capabilities in healthcare, while Google’s Verily is dedicated to the life sciences and Alphabet’s Google Ventures is heavily invested in health. Apple has focused on consumer-focused health technology, including disease tracking and prevention. Amazon is entering healthcare on multiple fronts. Amazon has partnered with Berkshire Hathaway and JP Morgan to form their healthcare company Haven. Amazon has also shown significant interest in medical supplies and pharmacy services (with its acquisition of PillPack in 2018), among others. Microsoft is exploring how AI can transform healthcare, genomics, telehealth, cloud computing, and cybersecurity in healthcare, in addition to other health initiatives.

The first digital therapy was approved by the US FDA at the end of 2018 and was launched in the United States in January 2019: Sandoz and Pear Therapeutics’ reSET-O, for the treatment of opioid use disorder.³⁶ Also at the end of 2018, Otsuka Therapeutics and Click entered a collaboration to develop digital therapeutics for patients with major depressive disorder.³⁷

ISPOR continues to monitor the role that digitalization will play in healthcare delivery and practices. The Society’s ISPOR Europe 2019 conference in Copenhagen, Denmark centered its conference content on the digital transformation of healthcare. Its opening plenary session focused on the impact of healthcare digitalization; the second plenary discussed what digital healthcare systems will look like and what it will take to deliver the changes required; and the third plenary explored the opportunities big data will have for research and learning.³⁸

It is clear that digital technologies will transform healthcare in ways that we may not have begun to imagine. The field of HEOR will serve an important role in advising and informing which technologies will best serve the goal of improving health outcomes while doing so in cost-effective ways.

9



AGING POPULATION THE IMPACT OF AGING ON HEALTHCARE DELIVERY AND COSTS

The aging population remains a top 10 trend for 2020. It was the number 4 topic in 2019 and number 5 in 2018. The global demographic trend of an aging population indicates that this issue will have a long-term impact on healthcare delivery and costs for some time to come.

According to WHO, by 2020 the proportion of the world's population over 60 years will nearly double from 12% to 22%, and the number of people aged 60 years and older will outnumber children younger than 5 years. While this issue of "population aging" began in high-income countries, by 2050, 80% of older people will be living in low- and middle-income countries.³⁹

An article in *The Economist* has identified the year 2020 as the beginning of the decade of the "yold"—the "young old"—a term the Japanese use for people aged between 65 and 75. The article notes that the yold are more numerous, healthier, and wealthier than previous generations of seniors. The article also points out that even though the yold are healthier than previous generations, this demographic shift will require drastic changes in health spending.⁴⁰

With aging populations come higher healthcare costs. In Japan, where 28% of the population is already over 60 years old, the government spent \$138 billion (or 15% of its total expenditure) in 2018 for healthcare and nursing.⁴¹ As a global leader in "super-aging" (identified as nations where at least 21 percent of its citizens are aged 65 or

older), Japan has been leading the way in innovative approaches to address population aging. Digital healthcare is considered to become a key driver of new solutions to serve this population's needs. To rein in costs, Japan is encouraging digital startups that can provide ways for the elderly to access medical consultations and is putting more efforts into using robotics, sensors, and high-end information and communication technology services to create patient monitoring systems that minimize human and financial burdens. Additionally, its efforts to transfer paper records to digital systems aim to feed these data into AI analytics tools for better and more cost-efficient care.⁴²

Many issues converge with the trend of the aging population—increasing healthcare costs, insufficient numbers of healthcare professionals and caregivers, and the desire to help patients age at home, to name just a few. Much hope rests on the potential for digital health to address many of these issues. HEOR professionals will be important contributors to help solve these issues and identify the approaches that will best serve the aging population.

"An article in *The Economist* has identified the year 2020 as the beginning of the decade of the 'yold'—the 'young old.'"

10



PRECISION MEDICINE A GROWING FIELD FUELED BY DATA

Precision medicine remains in the top 10 for 2020. This trend was number 9 in 2019. Also referred to as “personalized medicine,” precision medicine aims to take into account individual variability in genes, environment, and lifestyle for each person.⁴³

The market for precision medicine is growing. It is expected to reach \$217 billion by 2028; driven by an increasing demand for personalized treatment, technological innovation and advancement (including biomarker-based tests/kits, next-gene sequencing, and precise imaging), as well as government support and regulations.⁴⁴

Precision medicine requires a colossal amount of data—big data and the ability to analyze these data play an important role. According to a 2017 white paper by Stanford University, 153 exabytes (one exabyte = one billion gigabytes) were produced in 2013 and an estimated 2314 exabytes will be produced in 2020, translating to an overall rate of increase of at least 48% annually.⁴⁵

As far as the impact of precision medicine on healthcare, according to the Personalized Medicines Coalition (PMC), in 2018, 25 personalized medicine approvals accounted for 42% of all new drug approvals by the FDA. PMC also identified a number of emerging trends related to the approvals of personalized therapies in their report, *Personalized Medicine at FDA*. These precision medicine trends included expedited FDA review; cancer indications

based on biomarker, not tumor type; the emergence of small interfering ribonucleic acid (siRNA) treatments; expanding indications; and the emergence of personalized medicine biosimilars.⁴⁶

In an October 2019 white paper, PMC and LEK Consulting reported that 55% of all oncology trials in 2018 involved the use of biomarkers, as compared with 15% in 2000. According to the report, this growth in the importance of biomarkers will have a major impact on almost all key stakeholders across the healthcare continuum, including payers, who will have to enable broad coverage for biomarker testing.⁴⁷

Precision medicine continues to be a topic of high interest at ISPOR. The Society's conferences have featured a number of sessions on the topic. ISPOR's Personalized/Precision Medicine Special Interest Group has been active for a number of years with the mission to develop good practices for outcomes research in the study design and utilization of genomics involved in personalized/precision medicine.⁴⁸ The intersection of big data and precision medicine points to a strong need for HEOR as precision medicine continues to evolve.

“Precision medicine requires a colossal amount of data—big data and the ability to analyze these data play an important role.”

REFERENCES

1. Center for Drug Evaluation and Research. *Submitting Documents Using Real-World Data and Real-World Evidence to FDA for Drugs and Biologics Guidance for Industry*. <https://www.fda.gov/regulatory-information/search-fda-guidance-documents/submitting-documents-using-real-world-data-and-real-world-evidence-fda-drugs-and-biologics-guidance>. Published May 2019. Accessed December 2, 2019.
2. National Institute for Health and Care Excellence. NICE explores extending its use of data to inform its guidance. <https://www.nice.org.uk/news/article/nice-explores-extending-its-use-of-data-to-inform-its-guidance>. Published June 25, 2019. Accessed December 2, 2019.
3. Cave A, Kurz X, Arlett P. Real-world data for regulatory decision making: challenges and possible solutions for Europe. *Clin Pharm Ther*. 2019;106(1). <https://ascpt.onlinelibrary.wiley.com/doi/epdf/10.1002/cpt.1426>. Accessed December 2, 2019.
4. ISPOR. Real-World Evidence. <https://www.ispor.org/strategic-initiatives/real-world-evidence>. Published July 30, 2018. Accessed December 2, 2019.
5. ISPOR. Real-World Evidence Transparency Initiative. <https://www.ispor.org/strategic-initiatives/real-world-evidence/real-world-evidence-transparency-initiative>. Published September 8, 2019. Accessed December 2, 2019.
6. ISPOR. Improving Transparency in Non-Interventional Research for Hypothesis Testing—WHY, WHAT, and HOW: Considerations from The Real-World Evidence Transparency Initiative. https://www.ispor.org/docs/default-source/strategic-initiatives/improving-transparency-in-non-interventional-research-for-hypothesis-testing_final.pdf?sfvrsn=77fb4e97_6. Published September 18, 2019. Accessed December 2, 2019.
7. Hearing on “Priced Out of a Lifesaving Drug: Getting Answers on the Rising Costs of Insulin.” <https://energycommerce.house.gov/committee-activity/hearings/hearing-on-priced-out-of-a-lifesaving-drug-getting-answers-on-the-rising>. Published April 10, 2019. Accessed December 2, 2019.
8. The US Department of Health & Human Services. American Patients First: The Trump Administration Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs. <https://www.hhs.gov/sites/default/files/AmericanPatientsFirst.pdf>. Accessed December 2, 2019.
9. H.R. 3 – The Lower Drug Costs Now Act. <https://www.speaker.gov/LowerDrugCosts>. Published October 2, 2019. Accessed December 2, 2019.
10. Witters D. Millions in U.S. lost someone who couldn't afford treatment. *Gallup*. <https://news.gallup.com/poll/268094/millions-lost-someone-couldnt-afford-treatment.aspx>. Published November 12, 2019. Accessed December 2, 2019.
11. Stein M. Food insecure Canadian households may have trouble affording prescription medication, study shows. *ACES News*. <https://aces.illinois.edu/news/food-insecure-canadian-households-may-have-trouble-affording-prescription-medication-study-0>. Published October 7, 2019. Accessed December 2, 2019.
12. Florko N. It's tiny biotechs – not big drug makers – that fear 'nuclear winter' from Pelosi's drug pricing bill. *STAT*. <https://www.statnews.com/2019/11/12/tiny-biotech-fear-impact-investment-pelosi-drug-pricing/>. Published November 12, 2019. Accessed December 2, 2019.
13. Dion K, Garner S, Grueger J, Schroeter M. Has the time come for Pharma to accept modest prices? Presented at ISPOR Europe 2019. <https://www.ispor.org/heor-resources/news/view/2019/11/04/has-the-time-come-for-pharma-to-accept-modest-prices>. Published November 4, 2019. Accessed December 2, 2019.
14. USFDA. FDA approves innovative gene therapy to treat pediatric patients with spinal muscular atrophy, a rare disease and leading genetic cause of infant mortality. News release. <https://www.fda.gov/news-events/press-announcements/fda-approves-innovative-gene-therapy-treat-pediatric-patients-spinal-muscular-atrophy-rare-disease>. Published May 24, 2019. Accessed December 2, 2019.
15. USFDA. Novel Drug Approvals for 2019. <https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2019>. Accessed December 3, 2019.
16. AveXis. What is Zolgensma? <https://www.zolgensma.com/what-is-zolgensma>. Accessed December 3, 2019.
17. Bach PB. Zolgensma: A remarkable new treatment, an ICER analysis, and a poorly justified price. *Health Affairs*. <https://www.healthaffairs.org/doi/10.1377/hblog20190617.50453/full/>. Published June 18, 2019. Accessed December 3, 2019.
18. ICER. A look at CFTR modulators for cystic fibrosis. https://icer-review.org/wp-content/uploads/2018/06/MWCEPAC_RAAG_060718.pdf. Published June 2018. Accessed December 3, 2019.
19. ICER. Cystic fibrosis. <https://icer-review.org/topic/cystic-fibrosis/>. Accessed December 3, 2019.

20. Ollendorf DA, Chapman RH, Pearson SD. Evaluating and valuing drugs for rare conditions: no easy answers. *Value in Health*. 2018;21:547-552. https://icer-review.org/wp-content/uploads/2019/09/Ollendorf-2018_Rare-Conditions.pdf. Accessed December 3, 2019.
21. Xu K, Soucat A, Kutzin J, et al. *Public Spending on Health: A Closer Look at Global Trends*. Geneva: World Health Organization; 2018 (WHO/HIS/HGF/HFWorkingPaper/18.3). <https://apps.who.int/iris/bitstream/handle/10665/276728/WHO-HIS-HGF-HF-WorkingPaper-18.3-eng.pdf?ua=1> Accessed December 3, 2019.
22. Shrank WH, Rogstad TL, Parekh N. Waste in the US health care system. Estimated costs and potential for savings. *JAMA*. 2019;322(15):1501-1509. <https://jamanetwork.com/journals/jama/article-abstract/2752664>. Accessed December 3, 2019.
23. Berchet C, Cecchini M, Couffinhal A, et al. *Tackling Wasteful Spending on Health*. 2017; OECD Publishing. <https://www.oecd.org/els/health-systems/Tackling-Wasteful-Spending-on-Health-Highlights-revised.pdf>. Accessed December 3, 2019.
24. Luhby T, Krieg G. *Health Care: How Plans From Biden, Buttigieg, Sanders and Warren Stack Up*. <https://www.cnn.com/2019/11/20/politics/democrats-health-care-plans-elizabeth-warren-joe-biden-pete-buttigieg-bernie-sanders/index.html>. Accessed December 3, 2019.
25. World Health Organization. *Universal Health Coverage*. [https://www.who.int/news-room/fact-sheets/detail/universal-health-coverage-\(uhc\)](https://www.who.int/news-room/fact-sheets/detail/universal-health-coverage-(uhc)). Published January 24, 2019. Accessed December 3, 2019.
26. Brugger U. *A Review of Coverage With Evidence Development (CED) in Different Countries: What Works and What Doesn't*. https://htai.org/wp-content/uploads/2018/02/CED_Report_Bruegger_Final_Version.pdf. Accessed December 3, 2019.
27. Pifer R. CMS clears Louisiana Medicaid 'Netflix model' for hep C drugs. *Healthcare Dive*. <https://www.healthcaredive.com/news/cms-clears-louisiana-medicare-netflix-model-for-hep-c-drugs/557696/>. Accessed December 3, 2019.
28. Montazerhodjat V, Weinstock DM, Lo AW. Buying cures versus renting health: Financing health care with consumer loans. *Sci Transl Med*. 2016;8(327). <https://stm.sciencemag.org/content/8/327/327ps6>. Accessed December 3, 2019.
29. Garrison LP, Towse A, Briggs A, et al. Performance-based risk-sharing arrangements—good practices for design, implementation, and evaluation: Report of the ISPOR Good Practices for Performance-Based Risk-Sharing Arrangements Task Force. *Value in Health*. 2018;16:703-719. [https://www.valueinhealthjournal.com/article/S1098-3015\(13\)01811-1/fulltext](https://www.valueinhealthjournal.com/article/S1098-3015(13)01811-1/fulltext). Accessed December 3, 2019.
30. Trump DJ. Executive Order on Improving Price and Quality Transparency in American Healthcare to Put Patients First. <https://www.whitehouse.gov/presidential-actions/executive-order-improving-price-quality-transparency-american-healthcare-put-patients-first/>. Accessed December 3, 2019.
31. Presser L. When medical debt collectors decide who gets arrested. *Big Story*. Propublica. <https://features.propublica.org/medical-debt/when-medical-debt-collectors-decide-who-gets-arrested-coffeyville-kansas/>. Published October 16, 2019. Accessed December 3, 2019.
32. European Commission. *Cross-border Healthcare*. https://ec.europa.eu/health/cross_border_care/overview_en. Accessed December 3, 2019.
33. Cretu F. *Cross-border Healthcare Directive Had Too Little Impact So Far, EU Auditors Warn*. *Euractiv*. <https://www.euractiv.com/section/health-consumers/news/cross-border-healthcare-directive-had-too-little-impact-so-far-eu-auditors-warn/>. Published June 7, 2019. Accessed December 3, 2019.
34. European Commission. *Pricing and Reimbursement of Medicinal Products*. https://ec.europa.eu/growth/sectors/healthcare/competitiveness/products-pricing-reimbursement_en. Accessed December 3, 2019.
35. European Commission. *Report From the Commission to the Council and the European Parliament Competition Enforcement in the Pharmaceutical Sector (2009-2017)*. https://ec.europa.eu/competition/sectors/pharmaceuticals/report2019/report_en.pdf. Accessed December 3, 2019.
36. Sandoz. Sandoz Inc. and Pear Therapeutics obtain FDA clearance for reset-O to treat opioid use disorder. News release. <https://www.us.sandoz.com/news/media-releases/sandoz-inc-and-pear-therapeutics-obtain-fda-clearance-reset-o-treat-opioid-use-0>. Accessed December 3, 2019.
37. Otsuka. Otsuka and Click Therapeutics collaborate to develop and commercialize digital therapeutics for patients with major depressive disorder. News release. <https://www.otsuka-us.com/discover/otsuka-and-click-therapeutics-collaborate>. Accessed December 3, 2019.
38. ISPOR. ISPOR Europe 2019 Plenary Sessions. <https://www.ispor.org/conferences-education/conferences/past-conferences/isporeurope-2019/program/plenary-sessions>. Accessed December 3, 2019.

39. World Health Organization. *Ageing and Health*. <https://www.who.int/news-room/fact-sheets/detail/ageing-and-health>. Published February 5, 2018. Accessed December 3, 2019.
40. Parker J. The decade of the “young old” begins. *Economist*. <https://worldin.economist.com/article/17316/edition2020decade-young-old-begins>. Accessed December 3, 2019.
41. Kunitachi, Onomichi. Japan tries to keep the elderly out of hospital. <https://www.economist.com/asia/2019/01/12/japan-tries-to-keep-the-elderly-out-of-hospital>. Accessed December 3, 2019.
42. How Japan’s aging society paves the way for the era of digital health care. *Forbes*. <https://www.forbes.com/sites/japan/2019/03/28/how-japans-aging-society-paves-the-way-for-the-era-of-digital-health-care/#32f0781a6e26>. Accessed December 3, 2019.
43. US National Library of Medicine. What is precision medicine? <https://ghr.nlm.nih.gov/primer/precisionmedicine/definition>. Accessed December 3, 2019.
44. BIS Research. Global precision medicine market to reach \$216.75 billion by 2028. News release. <https://www.prnewswire.com/news-releases/global-precision-medicine-market-to-reach-216-75-billion-by-2028-891830298.html>. Accessed December 3, 2019.
45. Stanford Medicine. Harnessing the power of data in health. *Stanford Medicine 2017 Health Trends Report*. <https://med.stanford.edu/content/dam/sm/smnews/documents/StanfordMedicineHealthTrendsWhitePaper2017.pdf>. Accessed December 3, 2019.
46. Personalized Medicine Coalition. *Personalized Medicine at FDA*. http://www.personalizedmedicinecoalition.org/Userfiles/PMCCorporate/file/PM_at_FDA_A_Progress_and_Outlook_Report.pdf. Accessed December 3, 2019.
47. Personalized Medicine Coalition. *The Evolution of Biomarker Use in Clinical Trials for Cancer Treatments*. www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/The_Evolution_of_Biomarker_Use_in_Clinical_Trials_for_Cancer_Treatments.pdf. Accessed December 3, 2019.
48. ISPOR. Personalized/Precision Medicine Special Interest Group. <https://www.ispor.org/member-groups/special-interest-groups/personalized-precision-medicine>. Accessed December 3, 2019.

DEVELOPMENT OF THE ISPOR 2020 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 primary 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, Chief Science Officer Richard J. Willke, PhD, and Associate Chief Science Officer Lucinda Orsini, DPM, MPH have led efforts in developing this list of the 2020 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 addresses 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 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 2020 Top 10 HEOR Trends included:

Topic Exploration

Comprehensive exploration to generate a “long list” of potential topics was conducted by examining HEOR-related 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 2019 conference held in May 2019 in New Orleans, LA, USA, 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 during its meeting at the ISPOR Europe 2019 conference held in November 2019 in Copenhagen, Denmark.

ACKNOWLEDGMENTS

The Society would like to thank all of the members of the Health Science Policy Council and its committees for their thought leadership and advisory role in guiding the development of the 2020 Top 10 HEOR Trends report.

ISPOR Health Science Policy Council and Associated Committees

Shelby D. Reed, RPh, PhD

Chair, Health Science Policy Council
Duke Clinical Research Institute
Durham, NC, USA

Policy Outlook Committee

Ola Ahmad Al Ahdab, RPh, PhD

Abu Dhabi, United Arab Emirates

Pamela Blumberg, MPH, DrPH

TriNetX
Fort Mill, SC, USA

Louis P. Garrison, Jr, PhD

The Comparative Health Outcomes, Policy,
and Economics Institute
Seattle, WA, USA

Jorge Gómez, MSc, PhD

GlaxoSmithKline
Victoria, Argentina

Jens Grueger, PhD

Boston Consulting Group
Zurich, Switzerland

Finn Børllum Kristensen, PhD, MD

University of Southern Denmark
Hillerød, Denmark

Eui-Kyung Lee, RPh, PhD

Sungkyunkwan University
Taipei, Taiwan

Newell McElwee, PharmD, MSPH

Boehringer Ingelheim
Ridgefield, CT, USA

Maciej Niewada, MSc, PhD, MD, MA

Medical University of Warsaw
Warsaw, Poland

Brian O'Rourke, PharmD

Canadian Agency for Drugs and Technologies in Health
(CADTH)
Ottawa, ON, Canada

Chris Pashos, PhD

Abbvie
Chicago, IL, USA

M. Suzanne Schrandt, JD

ExPPect
Washington, DC, USA

Redouane Soualmi, MSc, PharmD

Boehringer Ingelheim
Algiers, Algeria

Adrian Towse, MA, MPhil

Office of Health Economics
London, England, UK

Science/Research Committee

Ibrahim Al-Abbadi, PhD, MBA, BPharm

University of Jordan
Amman, Jordan

Michael F. Drummond, MCom, DPhil

University of York
York, England, UK

Ana Lucia Hincapie, MSc, PhD

University of Cincinnati
Cincinnati, OH, USA

Rok Hren, PhD

Hren & Partner
Ljubljana, Slovenia

Maarten Ijzerman, PhD, MSc

University of Melbourne and University of Twente
Melbourne, Victoria, Australia

Khalid Kamal, MS, PhD

Duquesne University
McDonald, PA, USA

Paul Kind

University of Leeds
Leeds, England, UK

Vivian Lee, PharmD

Chinese University of Hong Kong
Shatin, Hong Kong

Josephine Mauskopf, PhD

RTI Health Solutions
Research Triangle Park, NC, USA

Karen Rascati, RPh, PhD

University of Texas
Austin, TX, USA

Shelby D. Reed, RPh, PhD

Duke Clinical Research Institute
Durham, NC, USA

Mark Sculpher, PhD, MSc

University of York
York, England, UK

Marthinus Stander, MBA, MD

VI Research FZ-LLC
Dubai, United Arab Emirates

Task Force Review Committee

Federico Augustovski, MSc, PhD, MD

Institute for Clinical Effectiveness and Health Policy
Buenos Aires, Argentina

Marc Berger, MD

Marc L. Berger, LLC
New York, NY, USA

F. Reed Johnson, PhD

Duke Clinical Research Institute
Durham, NC, USA

C. Daniel Mullins, PhD

University of Maryland
Baltimore, MD, USA

Donald Patrick, PhD, MSPH

University of Washington
Seattle, WA, USA

Scott D. Ramsey, PhD, MD

Fred Hutchinson Cancer Research Center
Seattle, WA, USA

Mark S. Roberts, MD, MPP

University of Pittsburgh
Pittsburgh, PA, USA



ISPOR

Improving healthcare decisions