

JULY/AUGUST 2021 VOL. 7, NO. 4

# VALUE & OUTCOMES SPOTLIGHT

*A magazine for the global HEOR community.*

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# VALUE & OUTCOMES SPOTLIGHT

JULY/AUGUST 2021  
VOL. 7, NO. 4

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

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## VALUE & OUTCOMES SPOTLIGHT PUBLISHING, SUBSCRIPTION, AND ADVERTISING OFFICE:

Value & Outcomes Spotlight  
Online: ISSN 2375-8678  
USPS: 019121

Published bi-monthly by:  
ISPOR  
505 Lawrence Square Blvd, S  
Lawrenceville, NJ 08648 USA

Direct photocopy permission and reprint  
requests to Director, Publications.

Cover photo courtesy of iStock.com/  
monkeybusinessimages

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## FROM THE EDITOR

As a child, I thought that everyone in their 40s was “old.” I saw many grandparents of my generation living in nursing homes which, at the time, was the norm and almost expected once someone became a certain age. When I became 40 years old, I could not believe time had passed so quickly, as I fundamentally felt much younger than the years implied. After all, age is but a number and should not be an indicator of how one expects to live one’s life. As a society, we need to “rethink” how we think about aging.

Today, more and more older adults want to stay at home and grow older in the comfort of their homes and communities. According to a 2018 Home and Community Preferences survey by the American Association of Retired Persons (AARP), approximately three-quarters of Americans who are 50 years or older want to reside in their own home for as long as feasibly possible, even if assistance is necessary for them to continue their daily activities. They want to be independent, but are concerned about their safety, mobility at home and around their communities, keeping their medical appointments, and participating in other daily activities that those of a younger age take for granted. For this population, these concerns are valid and should be addressed to better allow individuals to age in their own homes.

Studies have shown that, by addressing these issues and making the appropriate changes, the economics are improved because often the cost of care at home is lower than the cost of care in a senior living community or skilled nursing facility. On the other hand, aging in place may not be an option for adults who want to be independent but who are challenged with physical, emotional, or cognitive issues that can interfere with their daily activities and their ability to function independently. In these situations, nursing homes, senior living communities, or skilled nursing facilities may be better options. Long-term care systems are challenged when deciding on which services should be covered.

In this issue, Rapp and Swartz highlight implementing a value-based approach to living in place versus traditional long-term care that would promote coverage of care options that provide the greatest benefits to frail persons and their informal caregivers at the lowest cost to the system. The importance of the caregiver’s voice in healthcare, drug development, and value assessment should also not be overlooked. The article by Vurgun and colleagues points out that establishing family caregiver roles early on when developing treatment options provides a better understanding of and data collection around those roles; the improvements in both the health and well-being of the caregiver and patient; and the potential reduction in healthcare costs. More research is needed to show that aging in place can be beneficial by yielding cost-effective outcomes and better quality of life, reducing the burden on informal caregivers and realizing an overall reduction in medical spending.

Lastly, everyone at *Value & Outcomes Spotlight* would like to thank Laura T. Pizzi, PharmD, MPH, who will be moving on from her role as Co-Editor-in-Chief and will be taking on another role within ISPOR as Associate Chief Science Officer. I want to personally thank Laura for her support and leadership as my Co-Editor and wish her the best in her new and exciting role.

As always, I welcome any and all input from our readers.  
Please feel free to email me at [zeba.m.khan@hotmail.com](mailto:zeba.m.khan@hotmail.com).

Zeba M. Khan, RPh, PhD

Editor-in-Chief, *Value & Outcomes Spotlight*



## ISPOR SPEAKS

## Visiting Old to Learn New

Isao Kamae, MD, DrPH, ISPOR President, 2021–2022, University of Tokyo, Tokyo, Japan

I am truly honored to be serving as ISPOR President for 2021–2022. As ISPOR's first President from Asia, it is a special honor for me to represent all members and perspectives within ISPOR. Many are accustomed to seeing the Atlantic-centric view of the world. I vividly remember when Federico Augustovski—the first ISPOR President from Latin America—addressed the membership, as he referenced “World Map Upside-Down,” the artwork that depicted Latin America at the top of the map. And of course, some of us—the Japanese and Asians—are more familiar with this Pacific-centric view of the world. This is another example of how the world can be viewed very differently, depending on your perspective and view.



Photo courtesy of iStock.com/ymgerman

As ISPOR President, I am an ambassador for all members and perspectives. I look forward to discussions with regional leaders from chapters, consortia, and networks. These members represent ISPOR in all corners of the world and have access to local information and feedback, which is essential as ISPOR continues to expand not only its geographic footprint, but its perspectives and priorities.

When I was running for ISPOR President and developed my vision statement as part of the election process, [Gauguin's painting](#), “Where Do We Come From? What Are We? Where Are We Going?” resonated with me.

#### Where Do We Come From?

ISPOR has been trusted and respected for more than 25 years as the global leader in health economics and outcomes research (HEOR). The first entity of ISPOR globalization was the [Asia Consortium](#) established in 2005. Its success has driven ISPOR to develop additional regional groups. The [Latin America Consortium](#) launched after the Asian model, with the [Central and Eastern Europe Consortium](#), [Africa Network](#), and [Arabic Network](#) following.

#### What Are We?

ISPOR is committed to its mission to promote HEOR excellence to improve healthcare decisions globally. Currently, we find the

Society's membership includes about 14,000 individual and chapter members representing more than 100 countries worldwide. Such great success for ISPOR is achieved by enhancing the organizational values with its 4 strategic pillars: Scientific and Research Excellence; Communication and Collaboration; Education and Training; and Member Engagement.



#### Where Are We Going?

ISPOR is continuing to implement its strategic plan to drive innovation in this new era of HEOR. I celebrate the [ISPOR Strategic Plan Update 2024](#) as it moves towards the next stage of its evolution. To make it a reality, many challenges remain in significantly changing environments of healthcare in the world. These include: innovative but expensive technology; artificial intelligence application; advanced bioengineering; aging society; global warming; and others. Working with the ISPOR community, we will challenge the strategic plan to find the best solutions for healthcare in the world.

Regarding those questions prompted by Gauguin, I present 6 key words using ISPOR for my theme as your President:

#### ISPOR Is Involved:

Its members stay involved in the global HEOR community through the Society. Our [virtual events](#) are leading scientific programs and through member participation, we strengthen ISPOR's ability to share leading research.

#### ISPOR Is Scientific:

The Society drives the strategic [scientific agenda](#) in the field of HEOR through a new science strategy and the involvement of thousands of experts.

#### ISPOR Is Professional:

ISPOR is the leading professional society for HEOR globally. We have a strong roadmap for the future that builds on the input of many stakeholders.

#### ISPOR Is Outstanding:

The Society supports outstanding achievement in the field of HEOR. Our [awards program](#) confers acknowledgment of leading work globally, expanded with a new award for members reflecting HEOR excellence in low- and middle-income countries.

**ISPOR Is Resilient:**

The Society has been driving innovation in the field for more than 25 years. Moreover, ISPOR is coming through a very difficult challenge as Jens Grueger, PhD, the former ISPOR President, launched the “new normal” with a digital program platform in the wake of the COVID-19 pandemic. The Society has taken advantage of opportunities during times of change and I am pleased to work with an outstanding Board of Directors and staff in maintaining this course.

Finally, I leave you with an old Japanese proverb with 4 Kanji characters:

## 温故知新

(pronounced “on-co-chy-shin”). It literally means “*Visiting Old; Learn New*,” which refers to the importance of learning the lessons of the past, while also embracing the future. As ISPOR and HEOR continue to drive innovation and shape the future of healthcare, we will be well served to remember this. •

**What you need...  
you know we got it:  
Respect of the global  
HEOR community.**



Leverage ISPOR's reputation and reach to maximize your visibility, impact, and connection within the HEOR field.

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# From Measuring Costs to Measuring Outcomes: Revamping Healthcare at a System Level

## Signal IN BRIEF

- There is a structural stalemate in healthcare, as arguments about value and outcomes are now driven by price considerations.
- The value argument should be driven around the production of health and outcomes, not cost and price.
- The pharmaceutical industry should not be excluded from the conversation and needs to rethink its business model to help deliver outcomes.
- Social determinants of health must be accounted for in the delivery of healthcare—from how patients receive medicines to how they can get to their appointments.
- HEOR research needs to be reframed on a community level to determine what outcomes should be measured.

Healthcare stakeholders are wrestling with how to define the value of treatments and therapies. The problem is that while value in healthcare can be defined in many different ways, depending on the perspective of the stakeholder, most of the decision making revolves around cost. This emphasis on price has made the pharmaceutical industry a favorite target of critics and reformers and has contributed to a “structural stalemate” that maintains an unsatisfactory status quo in healthcare.

John Singer, Executive Director of Blue Spoon Consulting and moderator of ISPOR's latest **Signal** episode that explored innovative approaches to how the industry views price and value in healthcare, summarized the problem this way, “With this structural stalemate, you’ve got the PBMs (pharmacy benefit managers) that are that are blaming pharma, and pharma is blaming the PBMs. And you’ve got *everybody* mad at the insurance companies. So, there’s this massive finger pointing, which is basically the structural stalemate.”

Singer maintains that addressing the unmet need in healthcare requires an entirely different narrative—one that captures pharmaceutical company input in a different way and is centered around the production of health and outcomes, not cost and price.

And while determining clinical outcomes needs to be part of the approach, it cannot be not the only driver. Pharmaceutical manufacturers that can figure out how to define the value a new product brings to the community—in addition to the clinical outcomes—can create shifts in competition and true innovation at the system level.

### Getting Into System-Level Strategy

In ISPOR's second **Signal** series event, “From Price Determining Value to Value Determining Price: It’s About Strategy at a System Level,” panelists debated about how to bring system-level thinking to healthcare and how the pharmaceutical industry, payers, and health economics and outcomes research experts can work together in a new system for commercial strategy. In addition to Singer, the panel was comprised of Alexander Billieux, MD, DPhil, Vice President, Social Determinants of Health, UnitedHealthcare Government Programs, Minnetonka, MN, and Michele Markus, Head of Global Health Accounts, Omnicom, New York, NY.

When talking about the composition of value and outcomes, the panelists were asked to consider whether there is a difference between price and cost, and how they are related to the production of health. In the United States, this preoccupation with healthcare cost dates back at least 50 years to the Johnson administration, and today the conversation still has not changed. The discussion focused on how we shift the narrative to the production of health being the basis of competition strategy.

## KEY TAKE-AWAYS

- The healthcare industry is wrestling with how to define the value of treatments and therapies. While value in healthcare can be defined in many different ways, depending on the perspective of the stakeholder, most of the decision making revolves around cost. This emphasis on price has contributed to a “structural stalemate” that maintains an unsatisfactory status quo in healthcare.
- There is a misalignment between the way care is paid for and the outcomes that are achieved. Current efforts to create ways of tracking outcomes and accountability in healthcare has created a system of process managers that contribute to administrative bloat. These legacy systems either need to be reformed or new infrastructures need to be designed from scratch.
- While value-based arrangements have demonstrated some success, to make the most of these deals, health plans must reach populations that have been marginalized. Health policies that consider social determinants of health (eg, distribution of wealth, influence, and power) together with innovative delivery arrangements for drugs and health services can help close the gaps within the healthcare system.
- It may be time to move away from quality as a measure and instead figure out how to try to measure interaction at a system level. The pharmaceutical industry is starting to move away from introducing products to introducing outcomes—and the HEOR field can help define what those outcomes should be. This is the kind of disruptive research that leads to disruptive innovation.

According to Billioux, the emphasis on cost is a symptom of continued dysfunction, and while spending continues to go up, outcomes are moving in the opposite direction. As a result, there is a misalignment between the way care is paid for and the outcomes that are achieved. “At the end of the day, health outcomes aren’t something that are readily defined in a consistent way, and it’s not something seen immediately,” Markus said. In other words, the issue is to create a system for personalized care and determine accountability in the healthcare system. While not advocating for a national healthcare system in the United States, Markus believes there must be accountability within each of the players currently involved.

Meanwhile, the current efforts to create ways of tracking outcomes and accountability in healthcare has created a system of process managers that contribute to administrative bloat. That raises the question of whether these legacy systems should be reformed, or if the “Gordian knot” should be cut and new infrastructures designed from scratch.

“We need to be modern in our metrics and have realistic expectations of what each of the players in the healthcare system can actually contribute,” Markus said. This means determining what is reasonable for each agency to contribute and what role everyone plays—from patients, to the pharmaceutical industry, to PBMs. “Right now, there’s a lot of finger pointing across the board but not a lot of end-to-end ownership, because no individual entity can own the end-to-end chain [in healthcare].”

Is there a practical way for pharma to collaborate with payers to address outcomes? Billioux noted that in states with innovative pricing arrangements, such as Oklahoma, Louisiana, and Washington, there was not “line editing collaboration” with pharmaceutical companies, but “clearly there were discussions before those policies went into place to test the waters on what value-based arrangements look like.” When Billioux was at the Louisiana Department of Health, he helped pioneer the state’s “Netflix” arrangement with Gilead for hepatitis C drugs. In other words, similar to the way Netflix viewers pay a flat rate per month to stream as many shows as they want, Louisiana contracted with Gilead to pay a flat fee per year for all the drugs needed to treat prisoners and low-income residents with hepatitis C in an effort to eradicate the disease in the state. Now these value-based arrangements are being talked about more broadly. “They’re still volume-based by default, but I think it leaves open the door for more outcomes-based risk arrangements,” Billioux said. In fact, United Healthcare is moving more of its pharmacy spend into value-based arrangements.

The panelists agreed that this shift into value-based spend means determining where pharmaceutical companies should be involved in healthcare delivery. “There has to be some acceptance from governments, from payers, and from everyone in the court of public opinion that pharma is able to engage in the conversation,” Markus said. The other part is looking at how modern healthcare delivery is transforming, as it’s not just payers, healthcare systems, and the pharmaceutical industry anymore. “We’re seeing the cohesion of a lot of former business models now being rethought, combined, and merging in different ways, whereby the traditional division lines that have occurred are no longer applicable,” Markus said.

Could the different industry entities—such as America’s Health Insurance Plans, Pharmaceutical Research and Manufacturers of America, and Pharmaceutical Care Management Association—come together to provide an industry-level shift in the economics in healthcare? Billioux believes that this is unlikely, pointing out that the only time recently when these organizations united was when the prospect of Medicare for All

came up. “I think it’s much easier to come together when you have a common enemy than if you really had to hash out where you have commonalities.”

With pharmaceuticals representing about 10% of medical spend and hospitals representing about one-third, the question is whether these groups can actually come together to try and build a coherent plan. This remains to be seen because there is still a lot of money to be made with the traditional business models.

### Social Determinants of Health and Pharmaceuticals

While value-based arrangements between pharmaceutical companies and states have shown some significant success, for the states to make the most of these deals, health plans must reach populations that have been marginalized. While social determinants of health have not been directly written into these arrangements, there are things such as

lack of transportation, which keeps patients from getting to clinics, that can impede care.

In light of this, pharmaceutical companies should consider innovative delivery arrangements for their drugs. Plus, as digital therapeutics and digital monitoring become more common, it is also important to consider the “digital divide,” which needs to be addressed throughout the healthcare system. “People who are difficult to engage are often very disadvantaged when it comes to digital access. How can we help ourselves by giving them that connectivity?” noted Billioux.

The COVID-19 pandemic highlighted shifts in communications and healthcare delivery. One example is how indigenous tribes were able to achieve high rates of vaccination by using their internal communications networks to inform their members. These are the types of networks healthcare providers and pharmaceutical companies could be working with, Markus believes. “We are moving from broad-based communications and delivery to individual-based communications and delivery,” Markus said. “We have to diagnose

getting access to care on a community and individual level, instead of making broad assumptions about where people are and how they can access the correct information to drive their care.” And new technologies should reach into those communities and the providers already there, rather than establishing new clinics.

### Reframing QALY Measurements and the ISPOR Value Flower

Big ideas coming out of China could be used as examples to shape conversations about value in healthcare. China has moved away from using gross domestic product as a measure of economic value for the country and instead is adopting a concept called global ecosystem product, where social determinants of health are elevated as a way to measure economic success or failure. In healthcare, perhaps it’s time to move away from quality as a measure and instead figure out how to try to measure interaction at a system level.

“There’s a lot not captured in QALYs and it comes down to community relevance,” Billioux said. For example, racism is one of those societal determinants in communities that often gets overlooked. “You have to redefine how you’re determining quality.” Additionally, QALYs tend to miss things such as the moments patients are really sick, or the consequences of aging, Markus pointed out. “They’re not really aligned to the moments that we need the healthcare system most.”

ISPOR’s Value Flower concept could be used to help realign health economics and outcomes research in what should be measured for outcomes—not just looking at a drug, but the infrastructure of care that surrounds how a drug is prescribed and used. This can be the way to measure system value.

“We need that kind of disruptive research to lead to disruptive innovation,” Billioux said. “The challenge of QALYs is they tend to take the system as it’s presented, and then build out and change things within the system. We really need that kind of innovative thinking



“There has to be some acceptance from governments, from payers, and from everyone in the court of public opinion that pharma is able to engage in the conversation.”

— Michele Markus



to say, 'What if we just reject the system and agree that measuring QALYs this way just inculcates the system?'" Billioux said.

Things such as structural racism and systems of discrimination are going to change the metrics external to therapies being tested and developed. The pharmaceutical industry is interested in helping define how value is defined beyond the pill, not only at the product brand level but at the company brand level. "One of the challenges is that when you're only viewed as causing a problem instead of being viewed as part of the solution, that

becomes very limiting in terms of your being able to impact the solution. Vilifying an entire subsector of the industry isn't going to get us very far," said Markus.

The pharmaceutical industry itself is starting to move away from introducing products to introducing outcomes. But then comes defining what those outcomes should be—it could be clinical benefit, or innovation, whether on a product level, or even a therapeutic category level. This would be a complete game changer and something we need to develop metrics for.

Markus alluded to the recent discussions that have been taking place around the approval of Biogen's

Alzheimer disease drug Aduhelm™, which has been criticized for being approved despite its less-than-stellar clinical outcomes. "Perhaps the biggest failure in the past 2 weeks has been around truly understanding the transparency by which the metrics were applied. We looked at clinical benefit, and the discussion has been by some members of looking beyond clinical benefit." While there are pathways of looking beyond clinical benefit, "it's how these pathways were implemented, when they were implemented, and how they were discussed that becomes the root cause of some of the problem," Markus says. The Aduhelm™ debate shows how cost remains a big determination of the innovation that a product represents. •



"We really need that kind of innovative thinking to say, 'What if we just reject the system and agree that measuring QALYs this way just inculcates the system?'"

— Alexander Billioux, MD, DPhil

### The Next *Signal* Event

ISPOR's next *Signal* event on September 28, 2021, will focus on, "The New Science of Cause and Effect: Causal Revolution Applied." The discussion will explore how causal models interact with data and are applied in HEOR studies. The panelists will address challenges of selection bias, personalized treatment effects, fusing data from several sources, and causality in observational studies.

### For more information and to register

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

### About the Author

Christiane Truelove is a freelance medical writer based in Bristol, PA.

## HEOR NEWS

**1 6 Guiding Principles for the Design and Use of Artificial Intelligence in Healthcare**

(JHEOR)

New guidance from the World Health Organization states that ethics and human rights must be central to the design, deployment, and use of artificial intelligence if it is to fulfill its promise of improving healthcare delivery.

[Read more.](#)**2 Tunisia Says Healthcare System Collapsing Due to COVID-19**

(Reuters)

With doctors overburdened and intensive care units full, Tunisia's health ministry warned that the healthcare system is in a "catastrophic" situation.

[Read more.](#)**3 Aspects and Challenges of Resource Use Measurement in Health Economics: Towards a Comprehensive Measurement Framework**

(Pharmacoeconomics)

A June 2021 study published in *Pharmacoeconomics* lays out a framework to comprehensively review methodological aspects of resource use management methodology in health economics and outcomes research.

[Read more.](#)**4 Medicines and Healthcare Products Regulatory Agency Outlines 2-Year Delivery Plan With "Patient First" Focus**

(PMLive)

The United Kingdom's Medicines and Healthcare Products Regulatory Agency has unveiled its delivery plan, spanning from 2021 to 2023, that promises to put patients first. This comes after a review by the Independent Medicines and Medical Devices Safety revealed a failure to listen to and respond to patients.

[Read more.](#)**5 Centers for Medicare & Medicaid Services Unveils Surprise Billing Rule: 10 Things to Know**

(Becker's Hospital Review)

The first in a series of rules to shield patients from surprise billing has been rolled out by the Centers for Medicare & Medicaid Services. Among other things, the rule now bans surprise billing for emergency services and protects against balance billing.

[Read more.](#)**6 The Future of Market Access: A New Model for the "Next Normal"**

(PRMA Consulting)

According to PRMA Consulting's Jeff Weisel, the new model of market access strategy should be built on 4 key pillars.

[Read more.](#)**7 4 Reasons to Watch the Patient Centered Outcomes Institute's Evolving Role in Healthcare Decision Making**

(Avalere)

Experts at Avalere contend that the Patient Centered Outcomes Institute's new proposed principles that lay the foundation of its research agenda will expand the organization's role in healthcare decision making.

[Read more.](#)**8 Walmart's Plan to Sell Another Private-Label Insulin Is Met With Skepticism**

(STAT News)

Walmart announced it would sell a private-label version of rapid-acting analog insulins for up to 75% off the cash price of brand-name products, but critics contend that the move will not really help the insulin price crisis.

[Read more.](#)**9 Prenatal Test Developed With Chinese Military Stores Gene Data**

(The Asahi Shimbun)

A prenatal test developed by the Chinese gene company BGI Group and the Chinese military is being used to collect genetic data, and is seen by the United States as a national security threat, according to Reuters.

[Read more.](#)**10 Dollar General Hires Chief Medical Officer as it Looks to Become a Healthcare Destination in Rural Areas**

(CNBC)

With the hiring of Dr Albert Wu as the company's first chief medical officer and plans to add more healthcare products to store shelves, Dollar General says it is responding to customers' requests for more convenient and affordable healthcare products and services.

[Read more.](#)

## FROM THE JOURNALS

## Living Longer, Healthier Lives in Limited Resource Settings

Section Editors: Soraya Azmi, MBBS, MPH, Beigene, USA; Agnes Benedict, MSc, MA, Evidera, Budapest, Hungary

Guest Contributor: Louise Parmenter PhD, MSc, IQVIA, Reading, UK

### Predictors of Health-Related Quality of Life Status Among Elderly Patients With Cardiovascular Diseases

Saqlain M, Riaz A, Ahmed AA, Kamran S, Bilal A, Ali H

*Value Health Reg Issues.* 2021; 24(C):130–140

Quality of life is an important patient-reported outcome in the care of older patients with chronic diseases owing to aging-associated limited physical activity and poor health status. The paper by Saqlain et al helps us to evaluate health-related quality of life (HRQoL), its predictors, and association with physical function among elderly cardiac outpatients in Islamabad, Pakistan. Here, healthcare resources are limited, and cardiovascular disease poses a challenging health concern, accounting for 19% of disease in those aged 65 years and older. This population has multiple manifestations of cardiovascular disease and most of the medications prescribed to treat their cardiovascular diseases are taken for a long period of time. To the best of the author's knowledge, this is the first study that assesses the HRQoL status among older cardiac patients in Pakistan. It is important to address the paucity of data concerning HRQoL in the older cardiac population in settings with low healthcare provision.

Health systems around the world are focusing on longer, healthier lives. Quality of life is imparted by a person's function and health status. Functional and health status become more dominant predictors of quality of life as we age. Health status measured by self-administered tools such as the HRQoL questionnaire provides a powerful forecaster of morbidity and mortality.

Physical function is often considered an important predictor of quality of life in the geriatric population. The physical functioning capacity of older individuals is frequently estimated by measuring their ability to perform activities of daily living. Activities of daily living can be defined as common everyday tasks that are required for maintaining an independent life or that are necessary for survival.

A descriptive, nonexperimental, cross-sectional study was carried out from May 2018 to October 2018 in the outpatient departments of a tertiary-care hospital. The population under study were patients aged >65 years with at least 1 cardiovascular condition. The EQ-5D-3L (Euro QOL) and Barthel index were used to measure the quality of life and performance of activities of daily living, respectively. The EQ-5D health questionnaire provides a simple descriptive profile and a single index value for health status. It measures the 5 dimensions of (1) mobility, (2) self-care, (3) usual activities, (4) pain/discomfort, and (5) anxiety/depression. The Barthel Index consists of 10 items that measure a person's daily functioning, particularly the activities of daily living and mobility. The items include feeding, transfers from bed to wheelchair and to and from a toilet, grooming, walking on a level surface, going up and down stairs, dressing, and continence of bowels and bladder.

A total of 386 patients were admitted during the study period, of which 52% (n = 201 of 386) were female and 80.1% (n = 309/386) were in the age group of 65 to 74 years. More than half (n = 233, 60.4%) of the patients were receiving polypharmacy (5-9 medications), and 11.6% (n = 46) were taking 9 or more drugs (excessive polypharmacy). According to Barthel index scoring, 70.5% (n = 272) of respondents were independent in performing daily living activities. Most contributors were educated to primary level (30.3%) or had no education (44.3%) and were low (44.8%) or middle (40.4%) income providing a valuable insight into an understudied population.

Mann-Whitney tests and Kruskal-Wallis tests are nonparametric methods that were used to determine the difference in the quality of life by the sample characteristics. Over half of the patients, 198 (51.3%) reported impairment in the mobility domain, and 272 patients (70.5%) indicated impairment in the depression domain. Mann-Whitney tests revealed EQ-5D scores and visual analogue scale scores significantly differed by the number of hospital admissions ( $P = .001$ ), fall history ( $P < 0.001$ ), and ADLs ( $P < .001$ ). Kruskal-Wallis analysis revealed that EQ-5D index value and visual analogue scale score were significantly lower among patients who had comorbidities and who were exposed to polypharmacy (5-9 medications) ( $P < .001$ ). In multivariate linear regression analysis, self-reported health ( $P = .006$ ) and performance of activities of daily living ( $P < .001$ ) were reported as influencing factors on health-related quality of life.

The paper could be an interesting read for anyone wishing to broaden their knowledge of the use of real-world evidence in healthcare settings with limited resources. Here, diseases often associated with high-income countries are having profound impacts. It goes beyond safety and effectiveness to illustrate where a better understanding of quality of life could support actions to improve many lives. Findings indicated poor quality of life, especially in the depression and pain/discomfort domains, among older patients with cardiovascular disease or heart diseases. Most respondents were low income, illiterate, unemployed, and with comorbid conditions and taking multiple medications.

Pakistan is a developing country with an increased burden of a geriatric population and a poor healthcare system with no special life assistance programs from the government, which made this population more prone to dilemmas that ultimately lead to poor quality of life. The authors suggest that measures should be taken to improve patients' perception and to enhance awareness regarding the importance of doing daily living activities as a predictor of good quality of life. The underlying structural problems will be harder to solve. •



## RESEARCH ROUNDUP

*Section Editor:* **George Papadopoulos, BSc(Hons), GradDipEpi**, Lucid Health Consulting and University of New South Wales, Sydney, NSW, Australia

*Guest Contributor:* **Aakash Bipin Gandhi, BPharm**, ISPOR Student Network Chair, 2019-2020, University of Maryland, Baltimore, MD, USA

*Welcome back to Research Roundup as we tackle the area of aging. We present recent research that highlights what aging contributes to healthcare costs; the importance of coordinated health and social systems to address the challenges of aging; what can be saved in morbidity, mortality, and costs; policy implications; and lastly, the impact of recent advances in the treatment of Alzheimer's disease and the challenges and implications. As always, we trust that you enjoy delving into the research of aging presented in this section and we look forward to highlighting new research in the next edition.*

### Actualizing Better Health and Healthcare for Older Adults

Fulmer T, Reuben DB, Auerbach J, Fick DM, Galambos C, Johnson KS. *Health Affairs*. 2021;40:(2) 219-225. <https://doi.org/10.1377/hlthaff.2020.01470>

#### Summary

By 2030, more people in the United States will be aged 65 and older than those aged 5 and younger. Our healthcare system is unprepared for the complexity of caring for a heterogeneous population of older adults—a problem that has been magnified by the COVID-19 pandemic. Here, as part of the “National Academy of Medicine’s Vital Directions for Health and Healthcare: Priorities for 2021” initiative, the authors identify 6 vital directions to improve the care and quality of life for all older Americans. The next administration must create an adequately prepared workforce; strengthen the role of public health; remediate disparities and inequities; develop, evaluate, and implement new approaches to care delivery; allocate resources to achieve patient-centered care and outcomes, including palliative and end-of-life care; and redesign the structure and financing of long-term services and supports. If these priorities are addressed proactively, an infrastructure can be created that promotes better health and equitable, goal-directed care that recognizes the preferences and needs of older adults. The paper makes 6 recommendations: (1) create an adequately prepared workforce; (2) strengthen the role of public health; (3) remediate disparities and inequities; (4) develop new approaches to care delivery; (5) allocate resources to palliative and end-of-life care; (6) redesign long-term services and supports.

#### Relevance

The authors propose a concerted, coordinated effort to advance 6 vital directions. Despite long-standing barriers to their adoption, the next administration and Congress—in partnership with state and local government entities—should tackle them with new vigor.

### What Can Economics Say About Alzheimer’s Disease?

Chandra A, Coile C, Mommaerts C. *National Bureau of Economic Research*. Working Paper No. 27760. Published August 2020. <https://www.nber.org/papers/w27760>

#### Summary

Alzheimer’s disease affects 1 in 10 people aged 65 or older and is the most expensive disease in the United States. The authors describe the central economic questions raised by Alzheimer’s disease. While there is overlap with the economics of aging, the defining features of the “economics of Alzheimer’s disease” is an emphasis on cognitive decline, choice by cognitively impaired

patients, and a host of issues where dynamic contracts between patients and caregivers are hard to enforce. There is enormous scope for economists to contribute to our understanding of Alzheimer’s-related issues, including drug development, efficient care delivery, dynamic contracting within the family and with care providers, long-term care risk, financial decision making, and public programs for Alzheimer’s disease.

#### Relevance

This National Bureau of Economic Research working paper discussion proposes that the “economics of Alzheimer’s disease” overlaps with a number of areas of economics: health economics; public finance; behavioral economics; experimental economics; family economics; mechanism design; and the economics of innovation, suggesting that the topic should attract almost all economists.

### Aducanumab for Alzheimer’s Disease: Effectiveness and Value: Evidence Report

Lin GA, Whittington MD, Synnott PG, et al. *Institute for Clinical and Economic Review*. Published June 30, 2021. [https://icer.org/wp-content/uploads/2020/10/ICER\\_ALZ\\_Revised\\_Evidence\\_Report\\_06302021.pdf](https://icer.org/wp-content/uploads/2020/10/ICER_ALZ_Revised_Evidence_Report_06302021.pdf)

#### Summary

The Institute for Clinical and Economic Review (ICER) revised an Evidence Report assessing the comparative clinical effectiveness and value of aducanumab (Aduhelm™, Biogen) for the treatment of Alzheimer’s disease. This updated version reflects changes made based on the breadth of the US Food and Drug Administration (FDA) label, the treatment’s announced price, and comments received from the manufacturer, patient groups, clinicians, and other stakeholders. While the evidence suggests that aducanumab’s side effects are both common and potentially serious, the 2 phase III clinical trials produced contradictory results as to whether aducanumab slows the progression of the disease or improves cognition. ICER has determined the evidence is “insufficient” to show a net health benefit for patients with mild cognitive impairment due to Alzheimer’s disease, as well as for patients with mild Alzheimer’s disease. ICER’s health benefit price benchmark range for aducanumab is \$3000 to \$8400 per year for patients with early Alzheimer’s disease. This range was slightly higher than what was included in ICER’s preliminary draft report, and the change is largely due to the FDA requiring fewer MRIs than what patients received during the clinical trials. ICER also calculated what a fair price would be for a hypothetical chronic maintenance therapy that halted the progression of dementia in people with Alzheimer’s disease.

### Relevance

We will continue to read a lot more about the cost-effectiveness of aducanumab and indeed future treatments for Alzheimer's disease, including engagement with a variety of stakeholders in a discussion of how we should reflect Alzheimer's disease value to patients and families, especially when there is uncertainty around a product's effectiveness, its price, and the scale of its potential use in the community.

### The Economic Value of Targeting Aging

Scott, AJ, Ellison M, Sinclair DA. *Nat Aging*. Published July 5, 2021. <https://doi.org/10.1038/s43587-021-00080-0>

### Summary

In this article, the authors argue that although the life expectancy for older adults may have improved over the past century, not all years of life gained may be healthy. Hence, healthcare planners may need to focus on improving aging by compressing morbidity (maximizing a healthy life span and minimizing time spent sick or disabled) as compared to extending life. The authors evaluate the economic value of targeting healthy aging as compared to increasing life expectancy for individuals by using the value of statistical life methodology. Specifically, this methodology allows investigators to determine the monetary value attributable to gains from increased life expectancy and improved health by evaluating an individual's willingness to pay to decrease the risk of death. The study was conducted from a US perspective. Information on number of deaths and years lost to illness were identified from the Global Burden of Disease dataset. Population-related birth estimates were identified from the US Census Bureau data. The authors found that compressing morbidity may offer higher economic gains to healthcare systems as compared to efforts focused on increasing life expectancy or eradicating individual diseases. For example, the authors found that focusing efforts on promoting healthy aging that can increase life expectancy by a year can result in economic gains worth \$38 trillion. Similarly, focusing on efforts that promote healthy aging and consequently increasing life expectancy by 10 years can result in economic gains as high as \$367 trillion.

### Relevance

Healthcare planners or policy makers may benefit from designing interventions that target healthy aging, which in turn can lead to increased life expectancy, improved quality of life, and cost savings in the future.

### Comparison of Healthcare Spending by Age in 8 High-Income Countries

Papanicolas I, Marino A, Lorenzoni L, Jha A. *JAMA Netw Open*. 2020;3(8):e2014688. [doi:10.1001/jamanetworkopen.2020.14688](https://doi.org/10.1001/jamanetworkopen.2020.14688)

### Summary

While it is well established that the United States has the highest healthcare expenditure globally, it is unclear how this healthcare spending differs across age groups and compares to populations of other high-income countries that have more homogeneous healthcare financing schemes. This cross-sectional study utilized data from the Organization for Economic Co-operation and Development to evaluate healthcare spending per capita by different age groups across 8 countries. This included the United States, Australia, Canada, Germany, Japan,

The Netherlands, Switzerland, and the United Kingdom. All estimates were standardized and expressed in terms of absolute US dollars. Expectedly, the mean per capita spending for the United States was \$9524 (1.9 times higher) than the mean per capita spending for the 7 comparator countries. Importantly, in term of age groups, this difference was the higher among individuals 65 years and older, especially among individuals in the 80- to 84-year age group (\$18,645). Additionally, Medicare-eligible beneficiaries in the United States had a 100% higher per capita spend on healthcare as compared to older adults in the comparator countries. Several factors could explain these findings, including differences in health status, prices of healthcare services, and breadth of covered services across the United States and the 7 comparator countries. Limitations of the study include the descriptive nature of the analysis that did not allow for control of differences inherent in the healthcare systems across the countries.

### Relevance

This study improves our understanding of differences in healthcare spending across the United States and other high-income countries by age group. Importantly, the findings related to the Medicare-eligible population in the study suggest that shifting to a Medicare-for-all healthcare system in the United States may not be the ideal solution to reduce substantial healthcare costs.

### The FDA's Approval of Aduhelm: Potential Implications Across a Wide Range of Health Policy Issues and Stakeholders

*Health Affairs Blog*. Published June 10, 2021. [doi:10.1377/hblog20210609.921363](https://doi.org/10.1377/hblog20210609.921363)

### Summary

On June 7, 2021, Aduhelm™ was approved by the FDA for treating Alzheimer's disease based on its ability to reduce amyloid plaques (a surrogate endpoint). This occurred despite a majority vote by the FDA's own drug advisory committee against aducanumab's approval due to lack of evidence surrounding its clinical efficacy. Further, the broad FDA-approved drug label indicates that the drug can be used to treat Alzheimer's disease. This does not give any additional guidance on managing Alzheimer's disease among key clinical subgroups that were excluded from the trial.

In addition to this controversy, this decision also has several economic implications. Aduhelm has been priced at \$56,000 for an annual course of treatment, which can increase Medicare spending by \$10 to \$100 billion dollars, annually. Hence, Aduhelm adds to the list of drugs that are subject to a drug pricing debate in the country. On one side, while heavy investments in drug research and innovation may justify high prices, access to these medications may be endangered, particularly for seniors, due to high premiums set by insurance companies to offset these costs.

### Relevance

Aduhelm represents the FDA's first Alzheimer's disease-related drug approval in over 20 years. Despite the controversy regarding its efficacy, the approval of Aduhelm also has far-reaching economic consequences for patients given its high list price. These mainly include barriers to treatment access and elevated premiums that would help payers offset high costs. •

## Virtual ISPOR Conferences and Events

### Signal

### Signal

*September 28 | 11:00AM – 12:30PM EDT*

#### **The New Science of Cause and Effect: Causal Revolution Applied**

*Guest Speakers:*

- **Judea Pearl, PhD**, Professor of Computer Science and Director of the Cognitive Systems Laboratory, Samueli School of Engineering, UCLA, Los Angeles, CA, USA
- **William H. Crown, PhD**, Distinguished Research Scientist at The Heller School for Social Policy and Management, Brandeis University, Waltham, MA, USA

*October 26 | 11:00AM – 12:00PM EDT*

#### **Venture Capital Investment: Upstream Decision Making on Value in Healthcare**

*Guest Speakers:*

- **Tom Cassels**, President, Rock Health and General Manager of Rock Health's Advisory Services, San Francisco, CA, USA
- **Alyssa Jaffe**, Partner, 7wire Ventures, Chicago, IL, USA
- **Christina Jenkins, MD**, Venture Partner, Phoenix Venture Partners, LLC, New York, NY, USA

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**August 11-12 | 10:00AM – 12:00PM EDT**

#### **Applied Cost-Effectiveness Modeling With R**

*Faculty:*

- Jeroen P. Jansen, PhD, Chief Scientist, PRECISIONheor, Oakland, CA, USA and Associate Professor, Clinical Pharmacy, University of California, San Francisco, CA, USA

**August 31 | 9:00AM – 1:30PM EDT**

#### **Developing Decision-Grade Real-World Evidence**

*Faculty:*

- Sebastian Schneeweiss, MD, ScD, Professor of Medicine and Epidemiology, Harvard Medical School and Brigham & Women's Hospital, Boston, MA, USA
- Jeremy Rassen, ScD, President & Chief Science Officer, Aetion, Inc, New York, NY, USA
- Ashley Jaksas, MPH, Vice President, Science/Sr. Scientist, Aetion, Inc, Boston, MA, USA

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- Universal Health Coverage in Latin America: The Long Road Ahead
- Real-World Evidence: Is Latin America Ready?
- Health Technology Assessment: The Way Forward for Latin America
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<b>September 23</b>	<b>1PM EDT</b>	Sponsored and Presented by <a href="#">Evidera</a>
<b>October 28</b>	<b>10AM EDT</b>	Sponsored and Presented by <a href="#">OPEN Health</a>
<b>October 28</b>	<b>11AM EDT</b>	Sponsored and Presented by <a href="#">Optum</a>
<b>October 28</b>	<b>12PM EDT</b>	Sponsored and Presented by <a href="#">Evidera</a>
<b>October 28</b>	<b>1PM EDT</b>	Sponsored and Presented by <a href="#">IBM Watson Health</a>
<b>November 18</b>	<b>10AM EDT</b>	Sponsored and Presented by <a href="#">Certara Evidence and Access</a>
<b>November 18</b>	<b>11AM EDT</b>	Sponsored and Presented by <a href="#">Syneos Health</a>
<b>November 18</b>	<b>12PM EDT</b>	Sponsored and Presented by <a href="#">PRMA Consulting</a>
<b>November 18</b>	<b>1PM EDT</b>	Sponsored and Presented by <a href="#">Evidera</a>

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# The Benefits and Challenges of AGING IN PLACE



**T**he population of adults aged 65 years and older in the United States is growing at a faster rate than any other age group and is set to reach 70 million by 2030. As a result, it is paramount to shift the conversation towards the unmet needs and desires of aging adults and the changes in current reimbursement policies that would incentivize healthcare providers to address these needs. Data show that most of the elderly would prefer to age in place, that is, in their homes and communities. However, fragmentation between healthcare and social systems, including long-term care, is often the reason why such choices are ineffective and costly for the elderly and their healthcare providers. Increasingly more research is being conducted to show that synchronizing social service support programs and the healthcare system may yield cost-effective outcomes and reduce clinical burden and overall medical spending.

Laura N. Gitlin, MD, Professor and Dean of the College of Nursing and Health Professions, Drexel University, Philadelphia, PA and Eric Jutkowitz, PhD, Assistant Professor of Health Services, Policy and Practice, Brown University, Providence, RI, provided a deeper insight into the current challenges and policy issues older adults face that need to be addressed.

## Older Adults Prefer to Age in Place

Surveys have shown that the majority of older adults would prefer to age in place, whether that means aging in their long-term residence or the community in which they have spent most of their lives. However, experts have been redefining what it means. Gitlin argues that instead of aging in place, the conversation needs to be shifted to older adults aging in the *right* place depending on their social, physical, cognitive, and financial needs. “For the majority, it means aging in a place that is familiar to them, where they have created a routine, possess historical knowledge, and carry nostalgic memories of past experiences,” Gitlin said. However, 2 major obstacles currently prevent older adults from comfortably aging in their communities—their poor physical environment and the lack of primary care coordination assistance.

While many senior citizens are intelligent, assiduous, creative, and do not experience major cognitive impairments, their physical abilities are likely to decrease over time. In 2020, 40% of those aged 65 and older reported having trouble moving around,<sup>1</sup> and Gitlin points out that as they start experiencing mobility difficulties, such issues as going up and down stairs, lack of first-floor bathrooms, or having bathrooms that are too small to accommodate wheelchairs, can severely affect their independence to carry out their activities of daily living. In these instances, the housing itself becomes a huge barrier to aging in place. In fact, 1 out of every 5 falls among seniors cause a serious injury, resulting in more than 32,000 annual avoidable deaths and imposing more than \$50 billion in medical costs for public and private healthcare programs.<sup>2,3</sup>

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**Two major obstacles currently prevent older adults from comfortably aging in their communities—their poor physical environment and the lack of primary care coordination assistance.**

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Yet it is not just the physical challenge that poses a substantial financial burden for the healthcare system. Untimely and uncoordinated access to primary care visits also negatively affects payers, providers, and patients alike. For example, lack of transportation to and from a healthcare appointment and administrative assistance for those who live in the community can lead to increased need for acute care. In 2016, approximately 11%, (or nearly 2 million of all older adult emergency department visits were associated with ambulatory care-sensitive conditions. These are conditions that could have typically been managed in a primary care setting, thus, substantially lowering the overall costs of a care episode. In fact, the majority of admissions for ambulatory care-sensitive conditions were related to chronic conditions that could be controlled by primary care physicians if addressed in time.<sup>4</sup> Similarly, minor and relatively inexpensive home improvements and modifications have the potential to significantly reduce risk of falls and subsequently provide significant savings to the healthcare system.

## Disconnect Between Health and Social Services

Gitlin suggests that an elderly individual's home and his/her living environment should be seen as part of the health profile. Over the past few decades, researchers have worked on demonstrating the benefits of various technologies and services, (eg, assisted living technologies and patient navigator programs) that would allow for the elderly to age in place and reduce emergency department visits and hospitalizations.<sup>5</sup> Gitlin's work has also shown that providing coordinated occupational therapy, nursing, and home repairs to low-income, disabled older adults can result in fewer falls, reduced difficulty to perform activities of daily living, and improved health-related quality of life.<sup>6,7</sup> She explains that the concept of the intervention was very patient-centric: an occupational therapist assessed a patient's home environment to coordinate necessary home improvements. “We found that the benefit was huge, and that we were able to reduce mortality after just 6 visits. In other words, we were able to slow disability, if you will, and decrease health utilization,” she said.

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**Untimely and uncoordinated access to primary care visits also negatively affects payers, providers, and patients alike.**

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Currently, traditional fee-for-service Medicare is very limited in the types of long-term care services that the program provides to its beneficiaries. Typically, these services do not extend beyond traditional healthcare. Consequently, when it comes to long-term care for adults who want to age in place, the majority of the social services costs that they encounter must be covered out of pocket. Jutkowitz points out that many people spend down their assets and eventually qualify for Medicaid, the largest payer of long-term care services. “But even in these instances, many individuals still have to organize and coordinate everything themselves, because there is no mechanism for coordinating social or long-term care services,” he explained. Gitlin points out that there are pockets of programs (eg, Program of All-Inclusive Care for the Elderly), but not all older adults will qualify or know about them, and not all medical and health professionals even know to refer their families. This lack of knowledge creates underutilization of programs such as Medicare Wellness and puts many older adults in a disadvantaged position. To improve utilization and access to these services, innovative approaches must be created that can incentivize primary care providers to change the way they evaluate their elderly patients' health status.

## Primary Care Providers Should Be Incentivized

Both Gitlin and Jutkowitz admit that the current system is overly complicated and fragmented and is not helpful for the patients. Current financial disconnect often hinders further advancement of coordinated care. Developing, implementing, and maintaining a social services program requires continuous funding, but most of the time none of the savings encountered by the healthcare system are redistributed to these programs. “We have data showing that some of these community and social programs save money, reduce healthcare utilization, and

decrease nursing home placement,” Gitlin explained. “But while these programs result in societal cost savings and in savings for the healthcare systems, they do not kick back to the programs themselves.” This budgetary and structural disconnect between the 2 systems leaves most social programs in great need for financial support. “Particularly during the COVID-19 pandemic, many of the community-based programs have really been struggling,” she added.

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**Developing, implementing, and maintaining a social services program requires continuous funding, but most of the time none of the savings encountered by the healthcare system are redistributed to these programs.**

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One way to introduce a shift in what providers view as primary care for the elderly is to incentivize changes in the reimbursement models. Alternative payment models and accountable care organizations can be viewed as good value-incentive program model examples. Created over the past decade, these models include a network of healthcare providers that work on delivering coordinated, timely, and high-quality care to the beneficiaries of public and private healthcare plans. Jutkowitz explains that, “these are programs where the healthcare system is responsible for the care and cost of that care for individuals or groups of people. There are set targets and the healthcare system receives a proportion of savings if their spending can be reduced below these targets. As a result, these providers are incentivized to reduce high-cost healthcare services, and as part of the process, they could invest in social care programs or other interventions that help prevent these costly events.” The benchmarks for these models are not only set to create incentives for healthcare spending reduction, but are also established for quality of care and measured outcomes, often accounting for geographic and social risk-factor variation. In January 2021, there were 477 accountable care organizations registered under Medicare Shared Savings Programs with 10.7 million beneficiaries. However, while these programs are a promising step in the right direction, currently they provide care to only about 20% of the overall 65 and older population in the United States, leaving millions of aging adults without adequate care.

### Where Can HEOR Add Its Value?

Since many of the accountable care organizations, programs are still in their early stages, they are continuously being evaluated and improved. One of the major challenges that value incentive program models have encountered so far

has been establishing a reliable, small subset of standardized measures that would create benchmarks for quality of care and overall program effectiveness. As a result, interventions and studies that directly measure impact of coordinated long-term care are needed. Jutkowitz emphasizes that the perspective from which an intervention measures its cost-effectiveness (or any other outcome metric) is extremely important. He explains that even when a program shows significant benefits from a societal perspective, if the stakeholders of the project do not receive the results relevant to their objectives, a widespread implementation and funding of such interventions will be a challenge. For example, if we look from the perspective of a provider or an accountable care organization, then reducing emergency department visits and hospitalizations as well as subsequent costs is the desired outcome. Programs that fail to show these exact results, even if they provide other considerable benefits, might not get the deserved support. Therefore, Jutkowitz points out, interventions that account for multiple perspectives are needed to synchronize social service support programs and the healthcare system. •

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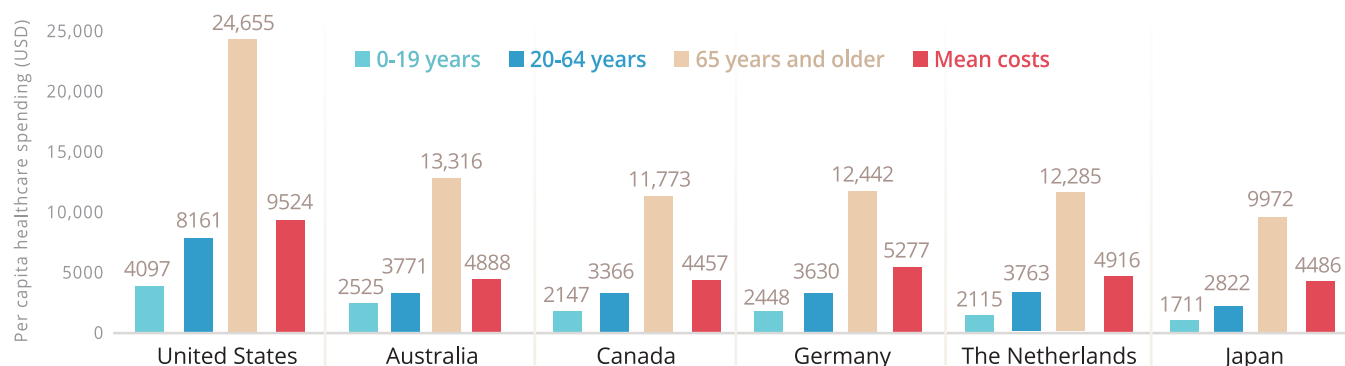


## By the Numbers: Aging as a Contributor to Healthcare Costs

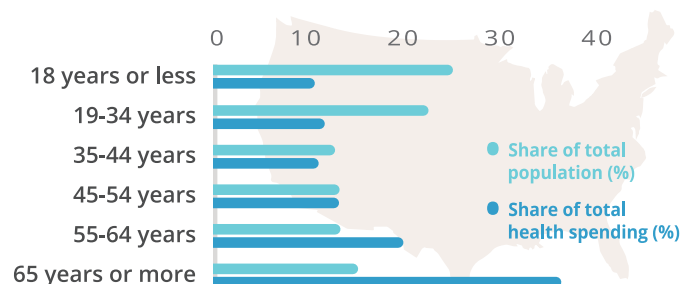
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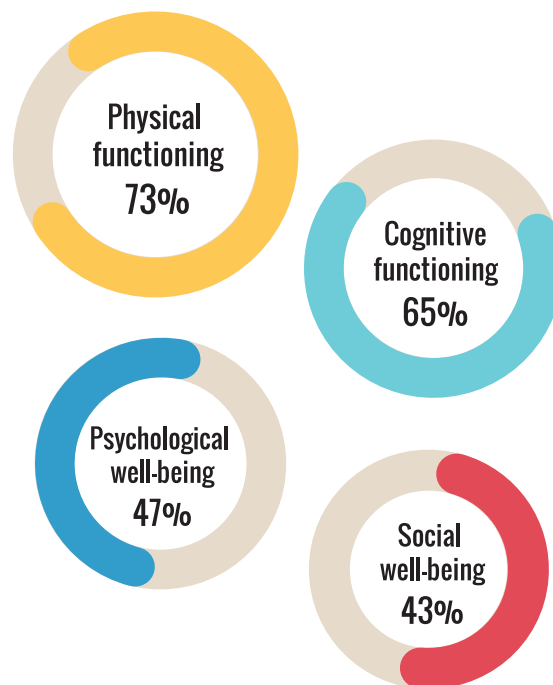
**Per capita healthcare spending (USD) by age group in 8 high-income countries, 2015.<sup>1</sup>**



**Distribution of healthcare spending by age group in the United States, 2016.<sup>2</sup>**

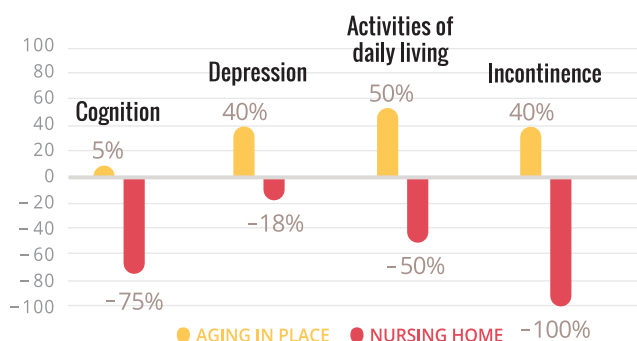


**Functional outcomes used to evaluate healthy aging in recent studies.<sup>4</sup>**



References available online.

**Clinical outcomes of aging-in-place care (home care and nurse coordination) vs nursing home care over 24-month period.<sup>3</sup>**



## Understanding Value in Cancer Care

Part 2 of a series exploring what value means to the stakeholders in healthcare.

Part 1 in this series can be found [here](#).

*Cancer caused almost 600,000 deaths in the United States in 2019, second only to heart disease<sup>1</sup> and more than the first year of the COVID-19 pandemic.<sup>2</sup> Most of us have lost someone to this devastating disease. Cancer cuts lives short at all ages. The very word frightens. It ravages patients' bodies and minds, causing pain and the loss of independence and dignity people often fear more than death itself. Cancer treatments cost an estimated \$209 billion in 2020 and this figure will continue to rise with the advent of more expensive treatments.<sup>3</sup>*



John Watkins, PharmD, MPH, BCPS

It's hard to overstate the impact of a cancer diagnosis. The emotional effect on the patient feels more invasive than the tumor, an all-consuming tidal wave that abruptly pushes everything else aside and penetrates all aspects of existence. Normal routines give way to surgery, chemotherapy, scans, labs, and other procedures as patients encounter a bewildering array of services, often poorly coordinated. Value in oncology involves not just the patient but a whole ecosystem that includes family, friends, and work associates and patients may need help to keep that ecosystem functioning.

Newly diagnosed patients face a series of complex and difficult choices they are unprepared to make. Tradeoffs are made. Life goals are reprioritized. Few anticipate the full impact of chemotherapy side effects. People tend to overestimate chances of survival and may choose a path they later regret.<sup>4,5</sup> Framing by providers affects choices. Patients need help to evaluate the choices and make good decisions.

### The Oncologist's View

Oncologists have subtly different views of what value means. Richard McGee, MD, has 4 decades' experience in community practice. For him, "Value is getting what you want from a treatment with the least cost and least penalty in side effects. What's difficult with the 'art of medicine' is that the cost for the patient is critically dependent on their life setting, not only on their medical history, but all the other things in their life, chronic diseases, age, families, and what things they value."

Evan Hall, MD, MPhil, an oncologist at the University of Washington, Seattle, WA, agrees that value is "mostly about individual patients." "High-value treatments' have benefits in the setting of cancer, usually measured in terms of prolonging life and hopefully improving quality, or at least not being a major detriment. "And then there is the cost," he adds. "Pharmacy costs and impact on patients' lives beyond their copay—logistics of getting to and from the cancer center, time in the chair, and days or weeks of side effects after treatment."

"I think about those mostly on the patient level," he continues, "but also at times at the population or system level—what's a high-value treatment for the health system in general. Sometimes things line up, but there's a little tension. Is this high value or not? It seems to have value to a specific patient, but not necessarily for the system. Some things I suspect are low value, but our evidence doesn't give us the answer."

This pinpoints a key problem. While drug prices are skyrocketing, clinical trial quality has decreased. Studies don't tell where a drug fits in treatment pathways. Should we treat sequentially or combine 2 or 3 drugs? In what order? Which strategy maximizes survival and quality of life? Value is uncertain when cancer drugs get accelerated approval with minimal evidence. Manufacturers get the desired indication, leaving the patient care team to figure out how to use them.

### The Patient's Journey

Learning details of a patient's life is the oncologist's first task. Patients omit things they think are unimportant, expect providers to disapprove of, or simply forget to mention. McGee says it's a process. "You can only get to that if you establish rapport, where you sit and chat about a variety of things. Over time you build a profile of their goals. Sometimes you ask directly."

**Value in oncology involves not just the patient but a whole ecosystem that includes family, friends, and work associates and patients may need help to keep that ecosystem functioning.**

Getting a diagnosis is very important to patients. "My initial reaction wasn't fear," recalls Tae, a long-time cancer patient. "We have a *name*. We know what it is—no longer an unknown thing. There was relief. Now we can start planning how to fight it!" Then an oncologist presents life-determining choices. Does the patient want to fight it? What is the patient willing to go through, risk, or give up? At age 17, Tae decided to fight. The chemo made her so sick she could only make it to school 1 day a week, but she persisted, and 35 years later, she's still swinging. "I just had something in me that—I'm not ready to give up." She has developed deep relationships with some amazing doctors. "I always felt the medical staff was working *with* me, not *on* me—giving me the power of decision. They've advocated very strongly at times for certain things. I had that network around me, but I was making the decisions. It's the patient's life. It's their body."

Hall uses the image of a scale: risks on one side, benefits on the other. "I can label things to help them understand should we do this or not? I put in reasons to do it and reasons not to do it. Sometimes dollars and cents come up. These things have costs.

If you're on active treatment, you'll probably hit your out-of-pocket maximum, but if you're just on surveillance, you probably won't. I usually present it in 2 dimensions—what we know about the benefits and what we know about the toxicities, including costs.”

“When you look inside yourself, what is it for you?” asks Tae. “What do you still want to do in this life, in this physical body that you have? It's not giving up hope. That doesn't mean that you fight way beyond when your body has said, ‘It's enough’ or when the doctors have said, ‘There's nothing more we can do for you.’ There's value in making that decision yourself—quality over quantity. What's important to you? What do you still want out of life?”

### Value is uncertain when cancer drugs get accelerated approval with minimal evidence. Manufacturers get the desired indication, leaving the patient care team to figure out how to use them.

Patients who want to survive will try aggressive treatments. McGee recalls a young woman with chronic lymphocytic leukemia. “I gave her information about the state-of-the-art (treatments) and her prognosis. She said very clearly, ‘I have two boys ages 10 and 7. I want to see them get to adulthood. Can I get there?’ That was her value. She would have been willing to do a bone marrow transplant despite the cost, discomfort, and risk. And 17 years later, when she was finally on her last legs, I reminded her of that conversation. She had this big grin and she said, ‘Yeah, you got me there. You got what I wanted.’” I remember a patient who chose the shortest regimen with the worst side effects. A mother of young children and a high school coach's wife, she needed to get it done and move on. Looking back, she said it was the right choice.”

Hall's balance helps patients understand how they're going to feel. “I incorporate things like quality of life. If people are not likely to be cured, I don't want them to feel worse from the treatment than from the disease. If somebody is asymptomatic, it might be right to treat them, but probably not to make them feel terrible with the treatment. Taking them from a relatively normal health state, even though they have metastatic cancer, and making them feel that bad is probably not acceptable. I try to empower patients if they're having toxicities to share with me, so we can adjust the plan. You try to get into their head and understand what they're trying to do.” Hall knows he is framing the discussion to some extent. “If you really think somebody needs to do a treatment, you present that in a slightly different way, even if you present the same facts. That's the art of medicine, helping people reach a decision, not just leaving them to fend for themselves in the sea of information and statistics.” Oncology nurses help by providing education and symptom management, helping patients and families through treatment, and facilitating communication with the oncologist.

A patient's value equation changes over time as they reach goals and set new ones. “The experience of living through the side effects changes the value as you go along,” McGee notes. “If they say, ‘Hey, I can't tolerate the neuropathy. It's too uncomfortable,’

you know the value equation has changed. I would say, ‘You know, this looks to me like you're having a hard time. Are you sure you still want to do it? We can alter the equations for you any time.’ You need to prompt them to think about it. Value statements are difficult, because they mean different things to each person and to the same person at different times, depending on their life setting. When regimens are equally effective, you select the least toxic or most tolerable. That's the dilemma faced by a highly competitive athlete diagnosed with testicular cancer. ‘If I take bleomycin, it will affect my lungs. I need them to perform as who I am. You can't take that away from me.’ He needed a different regimen without bleomycin in it.” McGee also recalls “a concert pianist who would not tolerate any neuropathy, so we had to eliminate vincristine and taxanes. It's also not uncommon for patients to select regimens based on their work schedules or other logistics.”

Nurse navigators and case managers help connect the services each patient needs. “Value for me is to make sure the patient received all their care from diagnosis to long-term follow up,” says nurse case manager Char Duffy, RN, Premera Blue Cross, Mountlake Terrace, WA, USA. “When they've just received the diagnosis, you have to get them resources, provide emotional support, and help them prepare for those appointments. Once they're really connected and get the feel of their treatment—once they have a treatment plan—people feel much more comfortable and they will move on to the oncology team. Then, they're tied in.”

“You can never tell how tough treatment will be for a new patient. I tell them to plan around it, having days off,” Duffy continues, “if you have it on Thursday, you have the weekend to recover. I try to help them understand that planning for fatigue and other symptoms is important before they start. The hardest thing is seeing somebody progress in their illness, and how the family changes throughout that process. That impacts you as a clinical person.”

Case managers address care gaps. “There are major places where the system fails people,” says Duffy. “I think the hardest thing is the length of time between appointments. They get a diagnosis and have to wait for the MRI. One patient had to wait 30 days for an oncologist. That is not good support. Assessment of supportive needs is often lacking.”

### End-of-Life Situations

End-of-life choices still matter. At the American Society of Clinical Oncology 2019, Atul Gawande, author of *Being Mortal*,<sup>6</sup> talked about Peg, his daughter's piano teacher. She had pancreatic cancer and canceled lessons. Gawande asked his “difficult questions:” What are your quality-of-life goals? What matters to you? What tradeoffs are you willing to make? Like Alex Trebek, Peg wanted to keep working. Teaching gave her life meaning. With home hospice, she taught for 6 more weeks and held a recital at which she gave each student a personal message. Peg made those 6 weeks count and her inspiration lives on in her students.

### The Importance of Holistic Palliative Care

Oncologists naturally lean toward the most effective treatment the patient will tolerate. To promote a positive attitude during treatment, the cancer patient culture uses militant language. We

had a “war on cancer.” Obituaries often say that patients “lost their courageous battle with cancer.” Organizations raise money to “fight” it. Sometimes the drive for aggressive treatment does not serve the best interests of patients unlikely to be cured, because it overlooks other long-term needs. Early referral for palliative care gives patients time and support to address emotional, spiritual, family, legal, and financial issues. Some patients fulfill aspirations involving travel. Palliative care seeks to achieve the best possible functionality for the longest time, a delicately balanced tradeoff. This, too, is more art than science, and it is less likely to happen when the focus is on survival at all costs.

Scott Ramsey, MD, PhD, Director, Hutchinson Institute for Cancer Outcomes Research, Seattle, WA, USA asks the key question. “Why do we place so little value on the end-of-life experience? Death is the one certainty, yet my experience as a researcher and a clinician tells me that we do a very poor job of preparing patients and families for this inevitable outcome, even when it is staring everyone in the face, and a worse job of managing it. If we spent 1 one-hundredth of what we spend on new drug research on new ways to manage end-of-life care, it would vastly improve its quality for everyone.”

### **Stereotyping: When a Patient Doesn’t Fit the Assumptions**

Some patients with cancer don’t fit the usual assumptions and this can affect how people, including providers, interact with them. “The very notion that a male can contract a ‘woman’s disease’ seems like a good example of the incongruous and unpredictable nature of humor,” writes Khevin Barnes, a male breast cancer patient. Humor helped him survive.<sup>7</sup>

Patients with breast cancer are sometimes criticized because people assume it’s “their fault” for having smoked. That’s unfair to any patient but moreso for patients who weren’t smokers. Frank Sierawski, age 34, had never smoked and had no family history of cancer, until his distressing cough was diagnosed as metastatic lung cancer. Now he promotes public awareness. “If there was ever an example of someone who never smoked and never thought they would get lung cancer, it’s me,” he says. “But the truth is anybody who has lungs can get lung cancer.” In some cases cancer diagnosis is delayed because providers aren’t expecting it.<sup>8</sup>

### **Financial Toxicity**

As cancer treatment costs increase, the burden on patients and society is becoming too heavy. “Most of the time the patient’s financial risk is limited,” says McGee. “Financial risk is borne by insurance, society, or other coverage systems. It’s extremely rare for a patient to bear the entire financial burden themselves, and so the cost issues become confused. If you’re buying my dinner, I’ll have a steak. If I’m buying it, I’ll have a hot dog.” For patients, the annual out-of-pocket limit becomes important. For the 2021 plan year, the limit on the public exchanges is \$8550 for an individual and \$17,100 for a family.<sup>9</sup> Hall says that cancer patients actively treated will reach these limits, which is a substantial part of most patients’ incomes. Researchers at the Duke Cancer Institute coined the term “financial toxicity,” defined as “the patient-level impact of the cost of cancer care.”<sup>10</sup> McGee says, “To the extent they want to get something done, especially if they’re young, patients will accept a lot of potential financial and physical toxicity that older people, perhaps wiser or more fatigued with life, simply aren’t willing to put up with.”

### **The Role of Payers: Achieving a More Collaborative Approach**

Payers must try to slow rising cancer costs. Some adopt stringent coverage criteria similar to those used in other drug classes, but this is less successful in cancer. Lack of evidence and patient heterogeneity are problematic. Information is lacking. Drugs have multiple indications—the US Food and Drug Administration-approved labels are a moving target. Guidelines encourage off-label use and regulators may limit payers’ ability to manage cancer drugs. A collaborative approach is needed, where payers engage with providers to develop more nuanced approaches to treatment. If providers consider total cost of care, not just patient out-of-pocket cost, payers can allow more latitude so individual patient treatment decisions remain with provider and patient, where they belong. •

*A single article can only skim the surface of this deep, complex, and emotional subject. Hopefully, this one encourages readers to explore on their own. Most of us have already been touched by cancer in one way or another, and it is almost certain that we will be again. Despite the unknowns, there is much that we do know that will help us care for others with cancer and make better decisions for ourselves, if needed. A cancer diagnosis is not the end of life, and for many, it leads to a richer and more meaningful experience of the time that remains.*

### **About the Author**

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# Implementing Value-Based Aging in Our Long-Term Care Systems

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Long-term care policies need to promote a value-based aging approach.

This approach needs to focus on providing services centered on those that are valued by people with care needs and their informal caregivers.

How can policies help older people make the most of these life-gained years, knowing that a share of those years may be spent with lower autonomy?

## Introduction

For the past 70 years, life expectancy in rich countries has increased by 15 years on average.<sup>1</sup> This situation raises a crucial question: how can policies help older people make the most of these life-gained years, knowing that a share of those years may be spent with lower autonomy?

While the implementation of value-based payment programs for healthcare has become a priority in most Organisation for Economic Co-operation and Development countries, long-term care policies are not currently focusing on value-based aging. This is surprising since long-term care policies for the past 2 decades have shifted towards the goal of “healthy aging,” the idea that reducing risks of disabilities that require costly long-term care services is the best strategy to contain the growth of future long-term care spending.<sup>2</sup> Healthy aging policies encourage providing home-based care services, which generally cost long-term care systems less than caring for a person in nursing facilities. Recognizing long-term care as involving choices between home-based and nursing home services calls for determining the value of different care options and then paying for services that have the greatest benefit at the lowest cost—the same objective of value-based payment programs for general healthcare.

**Long-term care systems should pay for care services that maximize older persons’ utility at the lowest cost possible.**

In this article, we further define the notion of value-based aging and show how it could be implemented in long-term care reforms to strengthen younger and older people’s interest in long-term care systems.

## The Concept of Value-Based Aging

The value-based approach to paying for healthcare combines (1) the use of patient-reported outcomes measures;

(2) a valuation of care resources used; and (3) the use of new technologies in different care options. The goal is to disclose the value of each of the care options to patients and care providers. This approach has been defined and formalized in abundant literature and promoted by the Organisation for Economic Co-operation and Development and the World Health Organization.<sup>3-6</sup> Under this approach, the value of care is measured by the incremental cost-effectiveness (cost-utility) ratio: an innovation or a service provides value to the patient if it provides more health benefits (quality of life) at a lower cost than a comparator or the standard of care. This approach favors an optimization of resources, allowing healthcare planners to focus on the services or innovations that provide the highest benefit at the lowest cost. Benefits are derived from patient-reported outcomes measures and therefore, take into account dimensions of health that are especially relevant to patients.

The concept of a value-based approach policy is similar: long-term care systems should pay for care services that maximize older persons’ utility at the lowest cost possible. Two features distinguish a value-based approach to long-term care. One is the extent to which it recognizes informal (unpaid) care provided by family and/or friends that enable individuals to live at home rather than in nursing homes. The second is that it acknowledges the indirect costs borne by the informal caregiver and the benefits that the caregiver may derive from providing care. In so doing, a value-based approach provides a framework for long-term care policies to cover some services that reduce particular burdens experienced by informal caregivers.

Following the standard set of outcomes measures developed by the International Consortium for Health Outcomes Measurement, one can consider that an older person’s satisfaction depends on 6 main dimensions: (1) place of death, as an older person’s preference is to die in his/her home; (2) the person’s

care experience, with well-being decreasing the more he/she receives polypharmacy or experiences falls; (3) quality of life, which varies negatively with pain, isolation, difficulties performing activities of daily living, lower mental and emotional health, and lower autonomy and control; (4) the perceived burden imposed on informal caregivers, which negatively impacts his/her well-being; (5) ability to participate in his/her healthcare decision making; and (6) clinical status, which reduces his/her well-being as he/she experiences frailty issues, spends time in hospital, and perceives his/her overall survival probability reduced.

Although different individuals weight each of these dimensions differently, they cover the most important drivers of older persons' utility function and include both objective and subjective measures of their quality of life. Indeed, while clinical status is an important component, subjective dimensions play a key role in defining the utility of long-term care services for older persons, with subjective well-being becoming a major determinant of frail older populations' use of long-term care. An important limitation of the International Consortium for Health Outcomes Measurement's approach, however, is that informal caregivers' perspective is not explicitly included; only the frail individual's perception of the burden he/she imposes on the caregiver is included.

In contrast, a value-based approach to long-term care can acknowledge the importance of the primary informal caregiver in the long-term care decision-making processes. Decisions to accept long-term care services usually are made by the person with long-term care needs and his/her primary informal caregiver(s) (family members, friends, or significant others). Given cost-savings for the long-term care system if a person remains at home, it is advantageous to maximize their joint utility rather than just the utility of the person with long-term care needs. While providing some care services can be difficult for a caregiver, considering only the perceived psychological or physical burden misses aspects that make the caregiver happier.

A value-based framework for long-term care policies expands consideration of

services that facilitate greater use of home-based care. In particular, non-medical services that reduce sources of anxiety might appear to be low value and yet be highly valued by a family trying to enable the person with long-term care needs to live at home. For instance, replacing a bathtub with a shower or providing a simple laptop computer and tutor to explain how to use telehealth and Zoom with friends might provide great value to such a family. Accounting for informal caregivers' utility also suggests that long-term care systems would provide greater value by covering difficult services in order to reduce informal caregiver burdens, thereby encouraging informal care for "quality tasks" that provide more satisfaction to the family caregivers. For example, helping to maintain catheters of any sort or change surgical dressings can be very stressful. If the long-term care system pays for professionals to deliver such services, informal caregivers can focus on tasks that provide greater value to the family such as those involving social interactions.

**Long-term care is rapidly shifting away from thinking that people with care needs are best served when they live in nursing homes.**

Note that a value-based approach for determining which long-term care services to cover involves a societal perspective, so only the costs that a long-term care system has to finance matter. Thus, although the informal caregiver's utility is included in calculating the value of different services, the monetary value (cost) of the caregiver's time is not taken into account because it is not reimbursed by the long-term care system. Others have estimated such costs, with the conclusion that the aggregate value of informal care exceeds the costs of formal care.<sup>7</sup>

### **Two Recommendations for Implementing Value-Based Aging in Long-Term Care Policies**

The value-based aging approach's advantage is that it prioritizes services

that bring the highest value to people with long-term care needs and their informal caregiver, and avoids paying for low-value services. This suggests re-evaluating which services are formally paid for by long-term care systems. For example, some tasks related to use and maintenance of medical equipment provoke anxiety among caregivers; more training would be valued highly by both the caregiver and the person with needs. Similarly, a caregiver's anxiety about helping a frail person bathe might be reduced if the long-term care system paid for a shower to replace a bathtub.

Second, value-based aging policies can be used to foster a "positive" aging perspective. By covering services that encourage older people to remain active and live at home, a value-based approach signals that they still have a role to play in society. Many people with mild long-term care needs fear losing contacts with friends or the ability to attend religious services once they or their caregiver can no longer drive, or public transportation is not an easy option. Covering some transportation expenses as long-term care services would promote active aging. Moreover, equating positive aging with a value-based approach may encourage younger people to consider how "senior years" can be well lived. To that end, a few countries (Canada, Australia, Norway, The Netherlands) have introduced "re-ablement" policies, which provide services designed to assist frail older people's needs rather than provide in-kind services. Instead of providing "meals-on-wheels," for example, these initiatives use physiotherapist services to help older people learn how to cook again by themselves, if cooking is an important occupation to them. A value-based aging policy would acknowledge the value of such re-ablement policies.

In conclusion, long-term care is rapidly shifting away from thinking that people with care needs are best served when they live in nursing homes. The issue facing long-term care systems now is how to decide which services should be covered. Implementing a value-based approach would promote coverage of care options that provide the greatest benefits to frail persons and their informal caregivers at the lowest cost to

the system. This might not necessarily be cost-saving in the short run, but it would ensure that resources are not wasted on low-impact services, and therefore could be an economically dominant strategy in the long run. New technologies, including mobile applications collecting self-reported questionnaires to detect needs and software platforms to improve information sharing among care providers, now provide great opportunities to implement value-based aging policies. While experimentations are needed to determine the benefits of these innovations in a value-based aging approach to long-term care, the question is, are policy planners ready to move forward? •

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# Time to Care: The Importance of Caregiver Voice in Healthcare, Drug Development, and Value Assessment

Nesrin Vurgun, PhD, Purple Squirrel Economics, a Cytel Company, Montréal, QC, Canada; Elizabeth Hubscher, PhD, and Karen Sandman, PhD, Purple Squirrel Economics, a Cytel Company, New York, NY, USA

In 2019, 703 million people aged 65 years or older were living worldwide.

There is a critical need for medical product manufacturers, value assessment agencies, and payers to improve engagement with family caregivers.

It becomes more difficult for caregivers to be a partner in care to the patient when they don't get the support they need.

## Introduction

Our global population is aging dramatically. According to the United Nations World Population Ageing report, in 2019, 703 million people aged 65 years or older were living worldwide—a number that is expected to double by 2050.<sup>1</sup> Older adults have traditionally received assistance from family members or other unpaid caregivers (hereafter referred to as family caregivers) with day-to-day activities as well as healthcare needs. Shifting demographics have wide-reaching implications: not only will more people need care, but caregivers themselves are getting older.

From a societal viewpoint, the value of informal care provided by family caregivers is immense. In addition to assisting older adults with activities of daily living, family caregivers are intimately involved with carrying out complex and wide-ranging healthcare activities. These include coordinating medical care, managing medications, monitoring symptoms, and performing direct patient care tasks. The AARP Public Policy Institute estimated that family caregivers provided 34 billion hours of unpaid care across the United States in 2017, which equated to an economic value of \$470 billion.<sup>2</sup>

The demands of caregiving, combined with limited formal training or external supports, can adversely impact the health and well-being of family caregivers. “Caregiver strain” is a concept that has been used to describe the spectrum of physical, psychological, social, and financial impacts experienced by family caregivers.<sup>3–5</sup> Dependence on family caregivers and high caregiver strain are most prominent in conditions that impose both physical and cognitive limitations, such as stroke, Alzheimer's disease, and dementia, which predominantly afflict older individuals.

C. Grace Whiting, JD, the president and CEO of the National Alliance for Caregiving, stressed that, “Family

caregivers are caring for multiple people ... [and] need care themselves to maintain their own health and wellness and to be the best care provider for a patient. When caregivers don't have the support they need, it becomes more difficult for them to be a partner in care to the patient.”

**Family caregivers provided 34 billion hours of unpaid care across the United States in 2017, which equated to an economic value of \$470 billion.**

Given the inherent societal value and costs associated with informal caregiving, there may be benefits associated with medical products that diminish severity of symptoms, slow disease progression, or lower treatment burden, thereby reducing caregiver strain. At present, there is a critical need for medical product manufacturers, value assessment agencies, and payers to improve engagement with family caregivers.

## Giving a Voice to Family Caregivers in Medical Product Development

Caregiving activities and caregiver strain evolve over the course of the care recipient's illness and depend on the care plan. As a result, family caregivers have unique perspectives into the burden of diseases, impact of existing treatments, and any unmet needs that could be fulfilled by novel therapies. In situations of medical complexity, cognitive impairment, or functional limitation, family caregivers can be essential participants during all stages of clinical development. They can facilitate engagement and participation in clinical trials and act as patient representatives. For example, in clinical trials of Alzheimer's disease or dementia interventions, family caregivers typically provide reports on the patient's cognition, behavior, global health, and functional status.<sup>6</sup>



Beyond the role of representative or advocate for the care recipient, family caregivers can also provide distinct information on their own well-being, strain, and health-related quality of life (HRQoL). These “caregiver-reported outcomes” can complement the patient’s voice as well as other efficacy and safety evidence. During clinical development, caregiver-reported outcomes data can be collected with validated general or disease-specific assessments.<sup>7</sup> One such assessment, the Zarit Burden Interview, evaluates caregiver strain in terms of physical, psychological, social, and financial well-being and has been validated in several languages for use in dementia. Caregiver HRQoL (eg, utilities) can be assessed with instruments such as the EQ-5D. These data can be incorporated as evidence in economic models to assess the cost-effectiveness of new medical products and ultimately employed by healthcare decision makers and payers.

### Informal Caregiving and Value Assessments

Inclusion of caregiver outcomes and costs of informal caregiving is essential to estimate the true impact of new medical products from the societal perspective, particularly for conditions with a greater demand for caregiving, such as Alzheimer’s disease or dementia. Many high-income countries (with the notable exception of the United States) have established national health technology assessment (HTA) agencies to assess the value of new medical products to patients, caregivers, and society. Evidence considered in HTA evaluations can include the so-called “spillover effect” of a condition, such as the family caregiver’s lost wages, productivity, or change in caregiver HRQoL.<sup>8</sup> The UK’s National Institute for Health and Care Excellence (NICE) recommends, but does not require, the inclusion of data related to the direct health of family caregivers (eg, EQ-5D data).<sup>9</sup> Nevertheless, a recent review of over 400 NICE technology appraisals found that family caregiver HRQoL was only considered in 3% of analyses; half of these involved pediatric patient populations and one examined Alzheimer’s disease.<sup>10</sup>

Economic benefits of new medical products may be underestimated in analyses that fail to encapsulate cost

offsets arising from the alleviation of caregiver strain, such as reduced productivity losses or decreased healthcare spending for the informal caregiver. In a review that examined cost-utility analyses published in Alzheimer’s disease or dementia, incorporation of caregiver-specific evidence (eg, HRQoL, time costs) generally led to better cost-effectiveness results than when these data were not considered.<sup>11</sup> The implications of omitting or inadequately capturing family caregiver-relevant outcomes in economic analyses and HTA submissions are serious and far-reaching, as they may influence access to new therapies and reimbursement decisions.

**Inclusion of caregiver outcomes and costs of informal caregiving is essential to estimate the true impact of new medical products from the societal perspective, particularly for conditions with a greater demand for caregiving, such as Alzheimer’s disease or dementia.**

One potential barrier to conducting analyses is the lack of published evidence on the economic burden of informal caregiving and disease- or population-specific family caregiver HRQoL or utilities data.<sup>10</sup> There are also limitations due to the oversimplification of what informal caregiving entails in analyses, such as the assumption that informal care is provided by a sole family caregiver.

According to Whiting, “Health assessors should think about social and behavioral determinants of health that can impact a family unit, such as financial costs (both long-term and short-term), emotional and physical strain due to care, and lack of support like other care providers.”

While HTA agencies such as NICE consider issues related to the equitable access of new therapies, equity in the

context of informal caregiving may not be captured in value assessments. Risk factors for high caregiver strain include socioeconomic factors such as social isolation, financial stress, longer hours of caregiving, and caregiving without a feeling of choice.<sup>3,5</sup> These risks are amplified for family caregivers from marginalized communities who face health disparities. For example, older adults from racial and ethnic minorities may rely on informal caregiving due to a variety of institutional, structural, and cultural factors, such as distrust, language barriers, limited resources, or lack of access to paid care.<sup>12</sup> In part due to these barriers, patients from these communities are also underrepresented in clinical trials or other health and economic studies, thus exacerbating family caregiver data gaps.

Whiting stressed that, “Medical product manufacturers must continue to include marginalized communities in the development of products that ultimately will be used by members of these communities. Medical product innovators should also ask what other social, economic, and environmental factors play a role in the participation of families from the communities they seek to include such as whether insurance will continue to provide healthcare if someone enrolls in a clinical trial; transportation to the trial site if necessary; expense of trial participation; cost of trial participation (and potential missed work); and cultural and language barriers that may make it difficult to participate without accommodation.”

### Looking Forward

The tremendous value informal caregiving brings to communities and societies often comes at a price for individual family caregivers. This price can only be expected to rise as the demand for informal caregiving increases along with a growing global population of older people. The complex reality of informal caregiving must be appreciated by medical product developers, healthcare decision makers, and other stakeholders. Engagement with family caregivers and collection of caregiver-reported outcomes with validated assessments should therefore be prioritized during medical product development as well as value assessment. Family caregivers are active

participants within the healthcare system and have a breadth of lived experiences. The distinct voice of caregivers must be recognized to appreciate fully the potential value of new medical products for individuals, families, and societies.

**Older adults from racial and ethnic minorities may rely on informal caregiving due to a variety of institutional, structural, and cultural factors, such as distrust, language barriers, limited resources, or lack of access to paid care.**

Whiting emphasized that, “Formalizing roles for family caregivers in medical product development allows us to better understand and collect meaningful data about the roles caregivers play in providing individual care, improving population health, and reducing healthcare costs. As medicine and healthcare continues to trend towards ‘whole person’ and ‘whole family’ approaches, medical innovators have an opportunity to build assessments that include what we know about family caregiving, leading to a more comprehensive understanding of the value of their products to families and the healthcare system.” •

### Acknowledgments

We would like to thank C. Grace Whiting and Lauren Rachel St. Pierre from the National Alliance for Caregiving (NAC) for illuminating discussions on caregiving in the United States. NAC is a nonprofit organization, which is dedicated to improving the lives of family caregivers through advocacy, innovation, research, and building partnerships with stakeholders. For further information, the reader is directed to the following research reports available from NAC:

- [Paving the Path for Family Centered Design: A National Report on Family Caregiver Roles in Medical Product Development](#) (May 2019)
- [Caregiving in the US 2020](#) (May 2020)

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# Add-On Payments and Price Evolution for Orphan Drugs in Oncology in Germany From 2011 to 2019

Mareike Konstanski, MSc, Thomas Mittendorf, PhD, Ulrike Kuchenbecker, PhD, Hanne Bubendorfer-Vorwerk, PhD, Xcenda GmbH, Hannover, Germany

Evaluation of add-on payments over time showed that it takes 4 years until a new oncology drug is sufficiently covered.

If an application is made too early, only a status 2 was granted, negating the possibility of negotiating extra funding.

Status is an important component for inpatient drugs to bridge potential reimbursement gaps.

In Germany, inpatient drug costs are reimbursed as part of the individual diagnosis-related group (DRG) payment rates. Since DRGs are being adjusted annually based on historic costs, there is a time lag between the point where a new technology is introduced into the market and when these costs are reflected in the DRG payment system. To address possible funding gaps for new and innovative treatments within the DRG system, hospitals can apply for extra budgetary reimbursement for a specific innovative technology for the subsequent year—the so-called Neue Untersuchungs- und Behandlungsmethoden (NUB).<sup>1</sup> The application period for a NUB is every year from the beginning of September until the end of October, a process that is led by the Institute for the Hospital Remuneration System (InEK) via an electronic tool. The InEK evaluates the applications and assigns a status that determines the eligibility for the negotiation of extra funding between a hospital and a health insurance (Table 1).

## Reimbursement for Inpatient Drugs in Germany

If the NUB status is 1, 3, or 4, hospitals can negotiate (add-on) reimbursement with the health insurances. NUB applications can be repeated yearly until

the cost for the new drug is reflected within the German DRG system.<sup>2,3</sup> For all newly approved drugs in Germany, the Federal Joint Committee (G-BA) assesses the additional benefit by conducting a formal assessment according to the Act on the Reform of the Market for Medicinal Products (AMNOG) as a basis for price negotiations to determine the drug's maximum reimbursable price. The National Association of Statutory Health Insurance Funds (GKV-SV) and the manufacturer will negotiate the final price, which is then applicable for the inpatient and outpatient settings. Because hospitals are autonomous in making purchasing decisions, manufacturers must negotiate twice. First, with GKV-SV to find the maximum reimbursable price to be paid within the Statutory Health Insurance (GKV) that will be listed in the public price tariff. Second, with the individual hospital or purchasing groups to determine the maximum purchase price, for which negotiated discounts are confidential.

The questions for this analysis were: (1) how NUB status changes over time for new and innovative drugs; (2) what is the timeframe from status approval until implementation into the DRG system; and (3) what implications it might have for the price.<sup>1,4</sup>

Table 1. Overview of possible NUB status.

Status	Definition / Decision Criteria
1	The innovation met all InEK criteria for a NUB (new, innovative; low number of patients, leading to a large cost variance in the existing DRG; higher average resource use as already covered by the DRG) and therefore fully accepted by the InEK. Hospitals subsequently have the possibility of negotiating a separate budget for the technology with health insurers.
2	The criteria were not met, leading to a rejection of a positive status. Hospitals are therefore not able to negotiate a separate budget for the new technology.
3	Submission of the NUB application after the due date (October 31). Hospitals may negotiate a separate budget even without the InEK decision. This status is not relevant in practice.
4	The InEK rated the submitted information as implausible and/or not verifiable, or the innovation was not yet available at the time of the final decision. Under exceptional circumstances, hospitals may also negotiate a NUB add-on payment on an individual basis.

*InEK indicates Institute for the Hospital Remuneration System; DRG, the individual diagnosis-related group; NUB, Neue Untersuchungs- und Behandlungsmethoden.*

We analyzed orphan drugs in oncology and the respective AMNOG dossiers submissions from the beginning of AMNOG until December 31, 2019.<sup>5</sup> InEK data were screened determining the result of the NUB applications.<sup>6</sup> The price history was derived from the official price tariff (Lauertaxe).<sup>7</sup> Only descriptive statistics were applied.

Since the beginning of AMNOG, 31 oncology drugs with orphan status were assessed (Table 2).

Within the first year of market entry, NUB status 1 was granted for 3 of the 31 products (~10%). In the second year, NUB status 1 was granted for 28 of the 31 (~91%) products. In the first year, 8/31 (26%) products received status 2 (denied), and 7/31 (23%) received status 4 (reimbursement under exceptional individual circumstances). For 13/31 (42%) products, no hospital applied for a NUB in the first year of market entry. The reason for this might be the timing of the launch date near or after the deadline for NUB submission and the likelihood of success of the NUB application. If an application is made too early, the data show that in most cases, only a status 2 was granted, negating the possibility of negotiating extra funding.

### NUB Status: Bridging Potential Reimbursement Gaps

For 7 out of 8 products with a status 2 in year 1, the status changed in the subsequent year (year 2). The remaining product received status 1 in the third year of application. All cases with a status 4 in the first year of application received status 1 in the second year. Changes from NUB status 1 to 2 are often accompanied by an integration of the drug into the DRG system through additional supplementary payments, or additional charges. Within the given timeframe, the mean duration of granted NUB status 1 and implementation into the DRG system was 4 years (range: 1–7 years) (Figure).

Ponatinib and pomalidomide maintained their NUB status for the longest period with a still ongoing status 1 for 7 years. The shortest duration was 1 year for Zalmoxis®. Of the analyzed drugs, 13 (~40%) had a status 1 for over 3 years at the time of this assessment. The observed mean duration of 4 years with

**Table 2. Orphan drugs with an oncology indication and complete AMNOG assessment.**

Active Compound	Trade Name	Start Benefit Assessment	NUB Status Year of Market Entry
<b>AMNOG Rating: Considerable Additional Benefit</b>			
Daunorubicin/Cytarabine	Vyxeos	01.10.2018	Status 2
Gilteritinib	Xospata	01.12.2019	-
Olaratumab	Lartruvo	01.12.2016	-
Pomalidomide	Imnovid	01.09.2013	-
<b>AMNOG Rating: Minor Additional Benefit</b>			
Cabozantinib	Cometriq	01.08.2014	Status 2
Decitabine	Dacogen	01.11.2012	Status 1
Inotuzumab ozogamicin	Besponsa	15.07.2017	Status 2
Ramucirumab	Cyramza	01.02.2015	Status 1
Ruxolitinib	Jakavi	15.09.2012	-
<b>AMNOG Rating: Non-Quantifiable Additional Benefit</b>			
Allogenic, genetically modified T-cells	Zalmoxis	15.01.2018	Status 4
Avelumab	Bavencio	01.10.2017	-
Axicabtagene ciloleucel	Yescarta	01.11.2018	Status 4
Blinatumomab	Blincyto	15.12.2015	-
Bosutinib	Bosulif	01.02.2014	Status 1
Brentuximab vedotin	Adcetris	01.12.2012	Status 4
Carfilzomib	Kyprolis	15.12.2015	Status 4
Daratumumab	Darzalex	01.06.2016	Status 4
Gemtuzumab ozogamicin	Mylotarg	01.09.2018	-
Ibrutinib	Imbruvica	01.11.2014	Status 2
Ixazomib	Ninlaro	15.01.2017	Status 4
Lenvatinib	Lenvima	01.07.2015	-
Midostaurin	Rydapt	15.10.2017	Status 2
Niraparib	Zejula	15.12.2017	-
Obinutuzumab	Gazyvaro	15.08.2014	Status 2
Olaparib	Lynparza	01.06.2015	-
Panobinostat	Farydak	01.10.2015	Status 2
Ponatinib	Iclusig	01.08.2013	-
Siltuximab	Sylvant	15.06.2014	-
Telotristat ethyl	Xermelo	15.10.2017	-
Tisagenlecleucel	Kymriah	15.09.2018	Status 4
Venetoclax	Venclyxto	01.01.2017	Status 2

AMNOG indicates the Act on the Reform of the Market for Medicinal Products; NUB, Neue Untersuchungs- und Behandlungsmethoden.

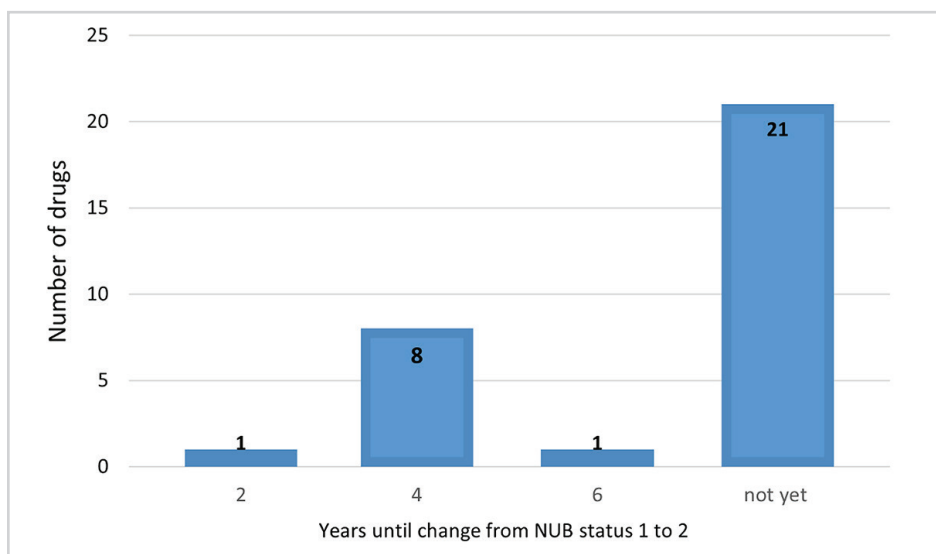
status 1 was longer than that assessed by Freiberg, et al in 2016.<sup>8</sup> The reason for this might be because the orphan status limits the available information for the yearly assessment by the InEK, causing longer periods of data collection.

For the majority (8/10 with NUB status change from 1 to 2) that lost status 1 over time, an additional fee was implemented. The 2 other drugs—Zalmoxis® and olaratumab—changed from NUB status 1 to 2 without the implementation of any additional

charges. Both Zalmoxis® and olaratumab are no longer available on the German market. Another drug lost its orphan status since the first assessment (ramucirumab) without consequences for the additional charges. Important events for price changes were (1) the AMNOG price negotiation and (2) change in NUB status. In the given timeframe, all prices (without considering discounts) decreased on average by 26% (range: 7%–44%), mainly after the AMNOG price negotiation or a change in NUB status. For granting a status 1, the price level



Figure. Time elapsed before a product loses a positive NUB status (change from status 1 to 2).



NUB indicates *Neue Untersuchungs- und Behandlungsmethoden*.

does not seem to be a key driver, as there was no relation between price and NUB status to be detected. Nevertheless, the funding of the related DRG and available data for the calculation seem to be influential.

### Conclusion

Even in orphan indications with only a limited number of patients, ensuring a NUB status 1 for new drugs is of paramount importance for hospitals to cope with individual high-cost cases.

The main factors of success for a NUB application are (A) the hospital perspective of economic consequences and (B) the stakeholder involvement to draft the application. •

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## Aging Effects and Promising Technologies

A conversation with Jerson Laks, MD, PhD

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Photo courtesy of Jerson Laks

# Q&A

*I spoke to Professor Jerson Laks, MD, PhD, an expert in dementia and neuropsychology and Coordinator at the Centre for Alzheimer's Disease and Related Disorders at the Universidade Federal do Rio de Janeiro, Rio de Janeiro, Brazil. He has authored or coauthored numerous papers on the subject of aging, including looking at how geriatric depression is tied to the development of Alzheimer's disease, the connection between early academic performance and dementia prevention, and burnout in caregivers of patients with Alzheimer's disease.*

“Dementia is underdiagnosed all over the world. We have a lot of work to do to improve dementia recognition in patients.”

**VOS:** Aging has been a major driver of resource consumption and healthcare expenditures. Can you talk about the rise in medical demands connected with aging, particularly dementia?

**Laks:** Dementia's 5-year incremental cost to the traditional Medicare program is approximately \$15,700 per patient. The longer the patient lives, which is, of course, one of the successful goals of the treatment, the more the cost increases because more comorbidities appear and costly measures have to be used.

**VOS:** Choosing the proper outcomes and measures for the health technology assessment is crucial. Please describe the best method for determining pharmacological benefits in people with cognitive impairments.

**Laks:** There is no medication to cure the process, that is, to prevent cognitive impairment from progressing. In fact, every available medication for Alzheimer's disease and cognitive impairment aims at slowing down the progression of the symptoms and signs. This occurs in about 35% of the patients using anticholinesterase inhibitors. We measure the efficiency of the medications, taking into account these results attained on a yearly basis, with minimum or low side effects. We do not measure positive effects, but rather, the lack of negative effects.

**VOS:** Elderly patients require well-coordinated advanced social and healthcare systems. What are some of the best international examples of successful healthcare and social systems, in your opinion?

**Laks:** No doubt, the Scandinavian countries and the United Kingdom are leaders in this field. The expenditures are based on the care provided and diagnoses are focused on the early cases in primary care. A whole series of programming and interconnected services for the elderly are then put in place.

**VOS:** What strategies are more effective in preventing age-related morbidity and mortality?

**Laks:** Controlling hypertension, diabetes, depression, weight gain in midlife, and stress, and using physical exercise. These measures are able to postpone the start of cognitive symptoms in Alzheimer's disease and also to control cerebrovascular dementia.

**VOS:** For the elderly, treatment and diagnosis are critical difficulties. Are you able to comment on these medical practices?

**Laks:** Dementia is underdiagnosed all over the world. We have a lot of work to do to improve dementia recognition in patients.

**VOS:** What factors have the most significant impact on the quality of life of the elderly?

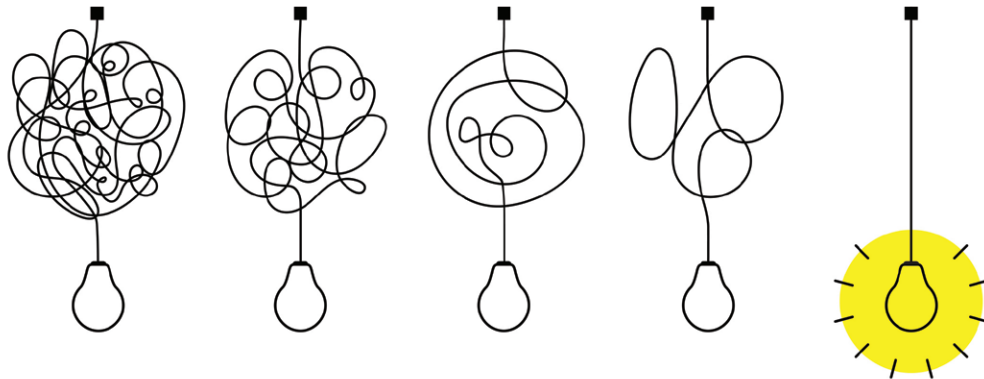
**Laks:** Physical independence is critical for quality of life in the elderly. Also, the ability to deal independently with things like bank accounts, driving the car, and other daily life activities so dear to the person are of importance.

**VOS:** Which technologies, such as medications, diagnostic tests, and other possibilities, are most promising for patients with Alzheimer's disease?

**Laks:** There is a push for early diagnosis of the disease at the preclinical phase. The goal is to diagnose the disease before dementia ensues. So far, a number of tests have been developed, but medication to treat the neuropathological and biochemical changes isn't available.

**VOS:** Do you have any experience with novel assisted-living technologies and how they might assist with home aging?

**Laks:** I have no personal experience with novel assisted-living technologies, which are expensive. Although I know about them and find them quite useful, the cost is certainly something to deter their use in Brazil. •



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