20 SENSE

THE 20SENSE REPORT

Spotlight on the Canadian Specialty Pharmaceutical Market

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Issue 22

Value-Based Healthcare in Canada

How the shifting paradigm will impact specialty medicine

Value-based healthcare: Defining the terms

Value in healthcare: The goal and the opportunity

INESSS's Dr. Michèle de Guise's bold vision for value in healthcare

Introducing Eva Villalba, Guest Editor for This Issue

The first Canadian to obtain a Master's degree in Value-Based Health Care (VBHC), Eva Villalba has a passion for public policy, community impact, healthcare reform, and social innovation. She is the executive director of the Quebec Cancer Coalition, serves as co-chair of the Resilient Healthcare Coalition (RHC) and is the Canadian ambassador for VBHC Center Europe. Given her pitch-perfect background in VBHC, we could not pass up the opportunity to invite Eva to guest-edit this issue. As expected, she brought a wealth of knowledge and expertise to the task.

Value-Based Healthcare: *Defining the Terms*

Elizabeth O. Teisberg and Michael Porter's book "Redefining Health Care: Creating Value-Based Competition on Results," published in 2006, ignited a global movement to measure value in health. While easy to grasp on the surface, the concept of value-based healthcare (VBHC) contains shades of meaning. Are we talking about value to the patient, the healthcare system, or other stakeholders? How do we measure the success of a healthcare system or intervention? To make things still more complicated, the American and European interpretations of VBHC have a somewhat different focus, and a few related terms sometimes get conflated with VBHC. The definitions below clarify the important concepts.

AMERICAN VBHC MODEL

Outcomes relative to costs (Michael Porter, Harvard University):
This model seeks to achieve "the best outcomes at the lowest cost,"[1] with "competition based on results" as the main driver of change. This requires shifting the focus from what physicians do to what patients need. Within this model, stakeholders implement VBHC through integrated practice units and care delivery, measurement of outcomes and costs, novel payment bundles, and robust technology to support service integration.

Outcomes that patients value (Elizabeth Teisberg, Value Institute for Health & Care, University of Texas at Austin):
This model places more weight on the quality of the outcomes than on cost containment, with an emphasis on "improving the health outcomes that matter to patients."[2] While these outcomes do not necessarily coincide with the outcomes most valued by doctors, hospital administrators, or health economists, what matters to patients ultimately creates value for all stakeholders.

Value Math*

$$\text{Value} = \frac{\text{Outcomes that matter to patients}}{\text{Costs throughout the patient journey}}$$

*Porter-Teisberg definition, adopted by the Canadian Institutes for Health Information in Canada[3]

EUROPEAN VBHC MODEL

Multidimensional value:
This model encompasses four distinct value goals:[4]
1) Personal value: helping patients achieve their goals
2) Technical value: achieving the best possible outcomes with the resources available
3) Allocative value: achieving fairness in healthcare delivery across patient populations
4) Societal value: acting as a positive force in social health and cohesion

RELATED TERMS

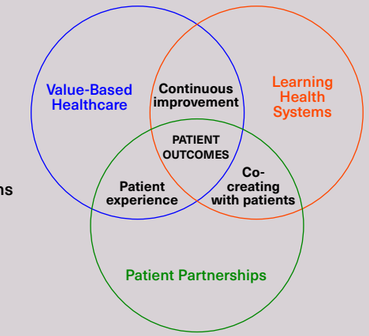
Patient partnership approach to care: This VBHC-adjacent term describes a fully collaborative model of care in which the patient and healthcare team work together toward the patient's health goals.[5] It reflects an ongoing evolution in the philosophy of care: from care **to** patients (provider-centric model) to care **for** patients (patient-centric model) and finally to care **with** patients.

Learning health system: Defined as "a health system that continuously improves," a learning health system builds improvement into its structure and operations.[6] The framework rests on a strong foundation of collaboration, with research, care, social services, management, and patients coming together to eliminate silos.

"Quintuple aim" for healthcare improvement: This integrated framework from the Institute of Healthcare Improvement engages all stakeholders in a health system to achieve improvement across five dimensions: patient health and experience, health equity, health of service providers, population health, and system efficiency.[7]

Putting it all together: Synergistic approaches to creating and improving value

- Value creation
- Use of data to drive decisions



Centring Value in Healthcare

To reach a goal, you need first to define it. So it's fair to ask: What is the goal of healthcare?

We might say it's simply to make people healthier. But healthier in what ways? For how long? At what cost to the system? And how do we measure the improvement?

Value-based Health Care (VBHC) addresses all these questions. VBHC seeks to improve health outcomes in meaningful ways throughout the care path. While it doesn't ignore costs, it steps away from the temptation to cut costs right now and instead looks at the whole cycle of care. It prioritizes prevention, early diagnosis, and timely treatment to reduce future spending on more advanced disease. It shifts the focus from delivering healthcare services (volume) to delivering health (value).

Within the sphere of specialty medicine, VBHC measures the total value of new medicines and acts on the information to benefit patients. It also establishes system-wide benchmarks, spots clinically unreasonable variations in practice, and takes steps to improve them.

WHAT VBHC IS AND WHY IT MATTERS

Back in 2004, a Harvard Business Review article called "Redefining Competition in Healthcare" helped plant the seeds of VBHC.[8] The co-authors of the article, Michael Porter and Elizabeth Teisberg, argued that a zero-sum competition mindset was weighing down healthcare. "Passing costs from one player to another, like a hot potato, creates no net value," they wrote. Instead, they invited stakeholders to "compete to be the best at addressing a particular set of problems." That way, the system would generate value, rather than simply dividing it.

Porter later distilled these ideas into a simple definition of VBHC: outcomes relative to costs.[1] Which outcomes, though? Teisberg addressed this question with a modified definition of VBHC – "outcomes that matter to patients."[2] To VBHC expert Christina Åkerman, this clarification makes all the difference. As she sees it, the problem in healthcare delivery and measurement lies in "not knowing what matters most." Before getting our yardsticks out, "we should therefore always ask ourselves: How well do we know the outcomes that matter most to the patients we intend to serve?"[9]

The European Commission widens the VBHC lens to include four distinct but overlapping pillars:[4] value to patients (personal value), making the best use of resources (technical value), distributing resources equitably (allocative value), and contribution of the healthcare system to societal well-being (societal value). This model could easily map onto the Canadian healthcare landscape and philosophy, which gives strong consideration to equity and social inclusion and more closely resembles European than American healthcare.

VBHC across the world

However we define it, VBHC is gaining prominence in the global healthcare agenda. In a 2021 white paper on healthcare systems innovation,[10] the World Economic Forum (WEF) recommends harnessing VBHC approaches, such as evidence-based modelling studies and outcomes-based agreements, to help capture the value of precision medicine and improve access to specialty medications with life-changing potential. Jurisdictions around the world are also using VBHC principles to guide broader health decisions and policies.

While American hospitals have been applying VBHC for years, international examples may resonate more with our Canadian healthcare system. In 2010, a group of 6 hospitals in the Netherlands formed an alliance called Santeon and adopted a VBHC strategy to improve patient care.[11] Starting with lung and prostate cancer, the consortium developed its own performance indicators and collected retrospective data going back 6 years. This led to an insight the group might otherwise have missed: the more expensive CT test for lung cancer didn't necessarily outperform standard chest radiography. On the flip side, robot-assisted prostatectomies perform better than traditional surgery. Rather than purchasing additional robots, the group saved costs by concentrating robotics expertise in a single hospital.

More recently, international experts from Academia VBHC used an instrument called the Atlas of Variation in Healthcare to expose unwanted and previously unnoticed variations in clinical practice in Brazil.[12] The loss of life due to cardiovascular disease, for example, varies by a factor of up to 5 among health regions, while the rate of Cesarean deliveries swings between 19.5% and 84%, a 4.3-fold variation. While some variation in outcomes is to be expected, an intervention that only works half the time signals a value problem, as does a high degree of variation between treatment centres or geographic regions. Brazil is now using the Atlas to guide its health policies.

THE VBHC OPPORTUNITY FOR CANADA

Here in Canada, we're seeing a growing appetite for the principles of VBHC, especially since the Covid-19 pandemic, which proved once and for all that a health system can surpass itself in a hurry. A report on VBHC readiness indicates that Canada has all three of the criteria needed to get VBHC up and running:[13] patient-centred care, payment models linked to quality, and standardization of healthcare quality.

With the pipeline of precision treatments putting increasing pressure on our health system resources, VBHC offers an agile and equitable approach to assessing medical innovation while ensuring access. VBHC also slides naturally into an interlocking health system like Canada's, in which improved outcomes, efficiencies, and cost-effectiveness benefit all stakeholders, including private payers.

Quebec's health technology assessment agency, INESSS, has put value at the centre of its strategic plan.[14] Not to be outdone, the province's Health and Wellness Commissioner has pledged to include relative value in its evaluation framework for long-term care facilities, enlisting no fewer than 71 performance indicators to the cause.[15] Individual centres are riding the same wave: in collaboration with the Quebec Cancer Coalition, Montreal's Jewish General Hospital is folding VBHC into a quality improvement initiative for colorectal cancer patients, with a similar model for people with lung cancer soon to follow in Quebec City.[14] Also in the provincial capital, the University of Laval Lung Cancer Institute has value-loaded the diagnosis and molecular testing of lung cancer, slashing the time between referral and treatment from the provincial mean of 56 days to just 26 days.[16]

The quest for value extends across the country. In 2015, the Canadian Institute for Health Information (CIHI) launched a PROMs [Patient Reported Outcome Measures] program and recently published a PROMs data collection manual for hip and knee arthroplasty.[17] New Brunswick's Primary Healthcare Integration Initiative aims for better collaboration among healthcare services offered in the community, from ambulance services to home care.[17] At Providence Health Care in Vancouver, British Columbia, VBHC is a key element in their Health Systems Redesign – an initiative which includes designing new physical spaces at St. Paul's Hospital opening in 2027, bringing teams together to improve integration of care across the patient's journey, and strengthening focus on what matters most to patients. Ontario has introduced bundled payment models that create incentives for integrated care, targeting such interventions as hip and knee replacement surgery and dialysis.[18]

"When looking at VBHC, hospitals represent the biggest opportunity," notes Jason Sutherland, a professor at the University of British Columbia's Faculty of Medicine.[19] No surprise, as "they're the biggest cost to the system." With up to 15 percent of hospitalized patients lacking the community support to get discharged, bundling hospital care with short-term home care seems a logical place to start.[19]

The pharmaceutical industry contribution to VBHC

The past two decades have poured extraordinary innovation into the specialty pharmaceutical landscape. Immune therapies that attack specific tumour cells, treatments for ultra-rare diseases, cell therapies that can regenerate diseased tissues... previously unimaginable treatments have become a reality, and the pipeline shows no signs of slowing down. While the new medications don't come cheap, they can save costs to the system over the long haul. At the same time, their promise outpaces the evidence typically required for HTA and payers, increasing uncertainty and leaving questions about their value despite their relevance to patient needs.

> "To design compassionate and meaningful care, you need to listen to the experts: people with lived experience."
>
> Researchers in a Canadian study of people with colorectal cancer

Applying a VBHC framework can help track the full value of these medications over time and in various health settings, allowing the strong performers to ride out the early data gaps and find their rightful place in the therapeutic arsenal. In this sense, VBHC can serve as an ally to pharmaceutical innovation.

Through collaborative partnerships and data-sharing arrangements with the public sector, industry can give the VBHC agenda a further push. The World Economic Forum has a few suggestions to get such partnerships off to a successful start:[20] 1) focus on outcomes that matter to patients, 2) engage partners across the whole health system, 3) anticipate challenges, and 4) focus on the problems within a particular health system, rather than cutting and pasting solutions from other jurisdictions.

MEASURING VALUE

Once we've defined value and agreed that we want it, we need to measure it. This starts with diagnostic testing, which can help identify the right patients for a complex treatment. "The importance of diagnostics tends to be underestimated in relation to therapeutics", says VBHC educator Dr. Marcia Makdisse.[21] But "a timely, correct and safe diagnosis process leads to early and appropriate care and enhanced recovery, impacting positively both outcomes and costs throughout the continuum of care." Poor diagnostic practices, on the other hand, can cause both testing complications and overuse of low-value treatments.

When measuring treatment outcomes, we need to establish what patients value most. Would that be overall survival? Reduced symptom burden? Rather than assuming, we can ask. A group of Canadian researchers did just that, in a study of people with colorectal cancer from across the country. The team used experiential tools to assess subjects' experiences across medical, physical, mental, and emotional dimensions.[22] What the researchers learned: during diagnosis, subjects placed the greatest value on compassionate communication and support from caregivers. During active treatment, meaningful discussions about options helped alleviate their anxieties. Those who recovered sought above all to regain a sense of competence and calm. The best way to design compassionate and meaningful care, the researchers concluded, is to "listen to the experts: people with lived experience."

For prostate cancer patients, fears of post-op incontinence and erectile dysfunction may rival or even outpace concerns about long-term health. In recognition of this reality, the Martini-Klinik, a specialty prostate cancer clinic in Germany, now uses such outcomes in evaluating the quality of its care.[23] Similarly, a lung cancer clinic in Belgium demonstrated that collecting patient-centred outcomes improved value by reducing emergency department (ED) visits and time spent in a day clinic, and even ensuring better quality of death.[24]

THREE PHARMACEUTICAL LEADERS SHARE THEIR THOUGHTS ABOUT VBHC

What does VBHC represent to a major pharmaceutical company? To Angela Behboodi, Government Affairs Director for Amgen Canada, it means "improving quality and outcomes, treating patients with the interventions that are best suited to them." Behboodi welcomes the paradigm shift. "VBHC requires us to rethink how we spend our health resources and ensure we incentivize outcomes over activities," she says. The current healthcare delivery model is largely reactive – what Behboodi describes as "break and fix." A value-oriented mindset also asks payers and policymakers to look beyond costs and toward better outcomes for all Canadian patients. Early and appropriate use of medicines can reduce the need for more expensive healthcare interventions down the line, easing demands on publicly funded resources.

"The right treatment, at the right time, based on each person's unique needs and preferences" is what value means to Cynthia Di Lullo, Pfizer Canada's Oncology Lead. "An outcome that's valuable to one person may have less value for another, and we need to capture that." In addition to addressing individual patients' needs, Di Lullo sees VBHC as a way to optimize resources across the healthcare system. "Better health outcomes and a better use of resources benefits all stakeholders over the long term." According to Di Lullo, VBHC aligns well with Pfizer's own vision of healthcare, and "a patient-centric mindset is embedded in everything we do."

Carlene Todd, Vice President of Access at Roche Canada, looks forward to public-private partnerships that will "truly bring industry to the VBHC discussion table." In her view, "sharing expertise and aligning on commitments to patients can only bring good things to all parties." While neither industry nor government can build a value-based healthcare system alone, "together we can go farther to give patients the outcomes they deserve, faster." Roche's strong focus on collaboration has led the company to support several platforms for change, such as the pan-Canadian Health Data Strategy Consultations and the pan-Canadian AI Strategy. "A continuous flow from data to evidence to insights is what enables us to create value," says Todd. It's also key to "building a resilient and sustainable healthcare system."

As a testament to the industry's commitment to creating value, these companies are participating in a multistakeholder VBHC demonstration project in colorectal cancer in Quebec and look forward to applying the learnings to drive best practices.

"A VBHC-oriented system rewards an outcome rather than a service, representing a profound philosophical shift."

Eva Villalba, Executive Director, Quebec Cancer Coalition and Canadian Ambassador for VBHC Center Europe

The long game

To align with VBHC, outcomes measurement needs to shift from our current focus on spending to a focus on continuous improvement. Instead of tallying up the costs of a medical intervention in isolation, we need to look at its impact on the patient and healthcare system over time. To this end, the Quebec Cancer Coalition's VBHC implementation project in colorectal cancer, spearheaded by Eva Villalba, seeks to establish a baseline cost for the whole trajectory of care relative to outcomes. "Governments *should* know how much the care path costs, but they're not actually measuring it," says Villalba. "They end up making decisions based on saving money on drug costs, which ends up costing more due to increased ER visits, complications, and rehospitalizations down the line. We're trying to change this short-term thinking."

The same reasoning leads Villalba to support the use of the DaVinci surgical robot, used for a range of procedures including prostate cancer surgery, gallbladder surgery and hysterectomy. "It's cheaper for humans to do the surgery as a one-off, but the robot provides more value because it performs the surgery more precisely and with fewer complications for the patient," she says.

The measurement of outcomes needs to zoom out to whole populations, capturing variations that may impact certain socioeconomic, demographic, or ethnic groups – such as the increased rates of diabetes in Indigenous communities or the poorer outcomes from Covid-19 in racialized communities. Teresa Tam, Canada's chief public health officer, acknowledges the need to "prioritize the collection and analysis of race-based data across the country."[27] In a similar vein, Villalba has been agitating for a detailed cancer registry to capture the added vulnerability to lung cancer in residents of Rouyn-Noranda, a Quebec town that sprang up around the Horne copper foundry.[28] Such data make it possible to create targeted prevention and treatment strategies for these communities.

The investment required to create and measure value calls for new funding and reimbursement models. Outcomes-based agreements (OBAs), for example, support value creation by tying reimbursement to outcomes. OBAs lend themselves especially well to potentially life-changing treatments that lack the traditional clinical evidence to establish value and satisfy payers. Treatments for rare conditions often fall into this category. OBAs can step in to fill the access gap while gathering real-world data to justify reimbursement – or not.

PROMS AND PREMS AS VBHC TOOLS

Patient-reported outcome measures (PROMs) have gained currency as an endpoint in clinical trials and other studies. PROMs focus on aspects of health that impact quality of life, such as ability to carry out day-to-day activities and mental health. A more recent category called patient-reported experience measures (PREMs) considers patients' perceptions of their care within the health system. PROMs and PREMs serve as powerful tools in the assessment of value and, in complement to clinical and administrative data, can help inform policy, programs, and value-based care delivery.[25]

The Canadian Institute for Health Information (CIHI) used PREMs to assess the performance of Canadian acute-care hospitals, focusing on how hospitalized patients perceived the coordination of care, treatment by nurses, and emotional support during their stay.[26] On this basis, they determined that 65% of patients had an overall "very good" hospital experience – encouraging, but with room for improvement.

PUTTING VALUE ON THE AGENDA

The advent of highly targeted specialty treatments has given new urgency to the goal of delivering "the right treatment to the right patient at the right time," and it's only by measuring value that we can get there. In deciding which outcomes to measure, we need to keep asking patients: "What is important to you?"

Our current system rewards volume – the delivery of health services (such as hernia surgery or chemotherapy), irrespective of value. A VBHC-oriented system rewards an outcome rather than a service, representing a profound philosophical shift. Putting appropriate incentives for improvement in place, and measuring what we want to improve, can help make it happen.

VBHC can propel us to the broader goal of a learning healthcare system, in which research, care, social services management, and population health join forces in a self-reflective and continuously improving network.[29] Rather than standing still, such a system remains in perpetual motion – "measuring, analyzing, taking action… and measuring again."[29]

The recent pandemic has called attention to the need for a resilient healthcare system. By using data to improve processes, outcomes, and efficiencies, VBHC supports this objective. With the momentum gained during Covid, Canada has an opportunity to put value at the forefront of its health-care system. **It's only by prioritizing value that we can give patients what they need: not health services, but health.**

In deciding which outcomes to measure,

we need to keep asking patients: "What is *important* to you?"

A Bold New Vision for Value in Healthcare

According to Dr. Michèle de Guise, INESSS is fully invested in the value agenda for health and social services

A "whole-person" philosophy permeates Dr. Michèle de Guise's approach to patient care, both in her former life as a practicing cardiologist and in her current position as President and General Manager of INESSS, Quebec's health technology assessment agency. Before landing at INESSS, Dr. de Guise implemented medical innovations in cardiology internationally, including at the Cleveland Clinic. She brought her passion for health promotion and patient experience to the Centre hospitalier de l'Université de Montréal (CHUM), where she served as director of health promotion, director of quality improvement, and later deputy director of professional services. INESSS's holistic mandate, which encompasses health technology assessment, health improvement, and social services, aligns with Dr. de Guise's vision of person-centred total health. As a passionate advocate for VBHC, she was glad to share her thoughts about how to create and assess value.

You have acquired a reputation as a leader and visionary at INESSS. Can you fill us in on your trajectory and current role?

I'm a cardiologist by training, with a particular interest in cardiac failure and heart transplants. At the CHUM, I opened an interdisciplinary cardiovascular rehabilitation clinic to help patients improve and prevent progression of their condition. A subsequent appointment as director of health promotion deepened my interest in health education. Under my direction, we integrated patient partners and patient experts into our care model. After the CHUM, INESSS felt like a natural step. I quickly learned about the challenges of assessing value in complex medications and other interventions where the value isn't always clear from the current evidence. I'm still learning!

Can you provide some background on INESSS's history and current mandate?

In 2003, Quebec had an agency called AETMIS to evaluate health technologies and interventions, and a separate agency, le Conseil du médicament, to evaluate medications. In 2009, AETMIS added assessment of social services to its mandate, and in 2011 AETMIS and the Conseil merged into INESSS. This level of integration makes INESSS quite unique among health technology agencies, but it makes perfect sense: people's experience doesn't divide into separate boxes. Another big milestone occurred in 2016, when we gained access to anonymized patient data from other Health Ministry databases and began assigning a unique INESSS identifier number to each patient. This has given our capacity to evaluate health technologies a giant boost: it means we don't just leverage data from the literature, but can see how new technologies play out in the Quebec

"Scientific innovation is happening so fast, we're constantly adapting to what comes next, even when we don't always know what 'next' looks like."

context. We're now looking to add hospital data to our repertoire. Imagine the rich knowledge that comes from integrating data across the cycle of care, from consultations with clinicians from various specialties, diagnostic exams, and medication use to ER visits, hospitalizations and mortality.

What's more, Quebec's life sciences strategy, which was put in place in 2017 and recently updated, enhances INESSS's role in evaluating innovations at various points in the life cycle. Over the past few years we have worked to strengthen our evaluation methods to ensure they can flex with specific innovations and methodological developments.

How do you define VBHC and what relevance does the concept have to INESSS and the Quebec healthcare system?

I see value as a better quality of care, better safety, and a better experience for the patient. A value-driven healthcare system also looks at health providers' well-being, which of course feeds into the quality of care for patients. Then there's the equity piece: how are people in remote regions going to access a sophisticated new treatment available only in a tertiary hospital in Montreal? In our province this is a constant concern. At INESSS, we aim to integrate all these aspects of value in our strategic plan. You could say we've put value in our DNA.

How do you see real-world data (RWD) intersecting with VBHC, today and in the future?

To make the most of a medication's value, we need to use it optimally within our healthcare system, which means giving it to the right patient at the right time, and RWD can support this aim. It can help us answer such questions as: How do results from clinical trials translate to patients being treated in the real world, who may not have the same health status or comorbidities as clinical trial subjects? Are we giving the medication to the right people? How does it compare to the standard of care in different patient groups? That's how we assess the value of a medication.

Does this mean restricting access to some innovations?

Once the value of a medication has been demonstrated, we should do everything in our power to facilitate access. But when a medication doesn't show value, we need agreed-upon mechanisms to stop recommending and using it. There's no point in giving a medication that doesn't offer value to a patient: it won't work and nobody benefits. Plus, it deprives the patient of a treatment that might work better. That's why we are moving towards a life cycle approach to health technology assessment, which emphasizes value across the entire treatment path and continuously generates and uses real-world data.

What are your thoughts about assessing precision medications?

To my mind, the big challenge lies in evaluating the field of precision medicine as a whole. We're not just talking about medications anymore: cellular therapies, for example, may have a drug identification number, but they're also complex treatments that have a bearing on the organization of healthcare and health services. We need new ways to conceptualize and assess such treatments. Scientific innovation is happening so fast that we always have to play catch-up. And we're doing it! We're constantly adapting to what comes next, even when we don't always know what "next" looks like.

Can you elaborate on the concept of patient partnership?

Medical treatment has become increasingly complex and we need greater and more varied expertise to inform decisions. Patients have a unique type of expertise because only they can tell us what it's like to live with, say, cancer, what they expect from treatment, and what they get from it. They can also help us understand the impact of side effects in relation to treatment benefits.

How do patients feel about taking on this enhanced role?

They're completely on board. And they show a remarkable understanding of the concept of value. Thanks to our efforts to integrate patients and the public into the drug evaluation process and to make our processes more transparent, patients have come to realize that the state can't take on *all* the risks for uncertain products. The risk ought to be shared. We also aim to communicate transparently with patients about medications with promising but uncertain value. That way, if they gain access to these medications, it's what I call "informed access." There is often a lot of uncertainty around the medium-to-long-term benefits and side effects of novel treatments, and patients need reassurance that there is a monitoring process in place to inform reimbursement decisions.

"How do we spread the risk so we harness the full value of the treatment, while also making adjustments if the value doesn't materialize as hoped?"

How ready is Quebec to incorporate VBHC at all levels of healthcare? Any obstacles you can identify?

I think we're in a good position for VBHC, partly because of INESSS. The term "value" has been democratized, and I sense a political will to incorporate the concept of value into our decision-making processes within the health system. What's missing is full data integration: the different silos aren't talking to each other yet, so it's still difficult to get a full picture of the patient journey. We're also weak on collecting patient-reported outcome measures – one of my preoccupations since my time at CHUM – as well as data on wait times and home care.

How would you like the specialty pharmaceutical space to incorporate value-based practices?

The rigour of industry-led RWD isn't quite where it could be. Ideally, the data should look beyond clinical outcomes and address the impact of a treatment on the healthcare system – things like the need for additional intensive care or staff training. I also see opportunities to ramp up comparative RWE, meaning evaluation of a new treatment against the standard of care. That's what regulators and payers are interested in. Comparing a treatment to placebo doesn't mean as much, because just about all medical conditions already have some form of treatment. Beyond efficacy, we need to ask: Do we have all the relevant information about the added value of a new therapy? Has our research methodology enabled us to capture this crucial information?

How would you like INESSS to use RWE in the future? Any limitations in the use of RWE?

There is a clear need for real-world data to complement randomized controlled trials – but it has to be high-quality data, and registry data may or may not meet that standard. Right now, the quality of RWE varies widely. Going forward, stakeholders need to apply quality standards to the data so they can use it to draw accurate and actionable scientific conclusions.

Sometimes, the nature of the treatment makes it difficult to collect data. Taking the example of a novel medication for a rare condition, it may take years to get enough data to assess the medication's value. Affected people don't have those years to wait, and sometimes you have to make a decision to give them access to the drug right away despite uncertainty about value.

Globally, outcomes-based agreements have been used to ensure timely access to costly medications and to distribute the financial risk of these treatments. How do such agreements integrate into VBHC and do they have a place in the Canadian specialty medicine ecosystem?

These mechanisms aim to distribute the risks associated with treatments that show promise but have as-yet uncertain value, so we don't delay access to patients. There is certainly a role for these treatments, but it's a challenge to determine how best to deploy them. The burden cannot fall solely on the state or the payer, and certainly not on the patient. The question becomes: how to spread the risk so we can harness the full value of the treatment – the right patient at the right time – and at the same time make adjustments if the value doesn't materialize as hoped?

In their current form, reimbursement agreements often incur a heavy clinical and administrative burden, with limited clinical evidence for the therapy in question. This makes it essential for stakeholders to work together to develop realistic and efficient frameworks for managing and mitigating the risks of certain innovations, while ensuring the agreements include clear direction on how to use the data collected to support them. The current shift toward a life-cycle approach to drug evaluation is a step in this direction.

What value-based directions and initiatives can we expect from INESSS in the months and years to come? What possibilities get you most excited?

I hope to strengthen our ability to respond to patient, clinician, and decision-maker concerns in a timely manner. The pandemic fast-tracked our agility and we don't want to lose that momentum. I'm especially excited about working toward a learning healthcare system – a system that constantly evaluates itself and adapts to new innovations and needs.

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Une nouvelle vision audacieuse de la valeur dans les soins de santé

Selon la Dre Michèle de Guise, l'INESSS est pleinement investi dans le concept de la valeur dans les soins de santé et de services sociaux

La philosophie de la « personne dans son ensemble » imprègne l'approche de la Dre Michèle de Guise en matière de soins aux patients, tant dans son ancienne vie de cardiologue praticienne que dans son poste actuel de présidente et directrice générale de l'Institut national d'excellence en santé et en services sociaux (INESSS), soit l'agence d'évaluation des technologies de la santé du Québec. Avant de se joindre à l'INESSS, la Dre de Guise a mis en œuvre des innovations médicales en cardiologie à l'échelle internationale, notamment à la Cleveland Clinic. Elle a perfectionné ses compétences en leadership au Centre hospitalier de l'Université de Montréal (CHUM), où elle a occupé plusieurs postes liés à la promotion de la santé et à l'accès aux traitements. Le fait que l'INESSS regroupe sous le même toit des activités d'évaluation des médicaments et des technologies, mais également des modes d'intervention en santé et en services sociaux, rejoint la vision de Dre de Guise en termes de santé globale et centrée sur les personnes. En tant que défenseure passionnée des soins de santé axés sur la valeur *(VBHC en anglais)*, elle était heureuse de partager ses réflexions sur la façon de créer et d'évaluer la valeur.

Vous avez acquis une réputation de leader et de visionnaire à l'INESSS. Pouvez-vous nous parler de votre cheminement et de votre rôle actuel?

Je suis cardiologue de formation, et j'ai un intérêt particulier pour l'insuffisance cardiaque et les transplantations cardiaques. Au CHUM, j'ai ouvert une clinique interdisciplinaire de réadaptation cardiovasculaire pour aider les patients à améliorer leur état de santé et à freiner la progression de leur condition. C'est dans le cadre d'une nomination ultérieure à titre de directrice de la promotion de la santé que j'ai approfondi mon intérêt pour l'éducation en matière de santé. Sous ma direction, nous avons intégré des patients partenaires et des patients experts à notre modèle de soins. Par la suite, une transition à l'INESSS m'a paru comme une étape naturelle après le CHUM. J'ai rapidement compris à quel point il était difficile d'évaluer la valeur des médications ou interventions complexes, dont le potentiel de valeur n'est pas toujours tangible dans les preuves scientifiques. Et j'en apprends encore!

Pouvez-vous nous donner quelques informations sur l'histoire et le mandat actuel de l'INESSS?

En 2003, le Québec comptait deux agences distinctes pour évaluer, d'une part, les technologies et les modes d'intervention en santé (l'AETMIS) et d'autre part, les médicaments (le Conseil du médicament). En 2009, l'évaluation des modes d'intervention en services sociaux s'est ajoutée aux activités de l'AETMIS, et en 2011, l'AETMIS et le Conseil du médicament ont fusionné pour former l'INESSS. Ce niveau d'intégration rend l'INESSS assez unique parmi les agences d'évaluation des technologies de la santé, mais c'est parfaitement logique : l'expérience des gens ne peut être compartimentée dans des boîtes distinctes. Une autre étape importante a eu lieu en 2016, lorsque nous avons obtenu l'accès à des données anonymes sur les patients provenant d'autres bases de données du ministère de la Santé et que nous avons commencé à attribuer un numéro d'identification unique de l'INESSS à chaque patient. Cette stratégie a grandement renforcé notre capacité à évaluer les technologies de la santé : cela signifie que nous ne mettons pas seulement à

« L'innovation scientifique est si rapide que [...] nous devons constamment nous adapter à ce qui vient par la suite, même si nous ne savons pas toujours à quoi cette "suite" ressemblera. »"

profit les données de la littérature, mais que nous pouvons voir dans quelle mesure les nouvelles technologies évoluent dans le contexte québécois. Nous cherchons maintenant à ajouter les données hospitalières à notre répertoire. Imaginez la mine de connaissances que représente l'intégration des données sur les parcours de soins depuis les informations sur les consultations de médecins de diverses spécialités, la consommation de médicaments, les examens diagnostics, les visites à l'urgence, le recours à l'hospitalisations, les décès. Par ailleurs, la Stratégie québécoise des sciences de la vie, mise en œuvre en 2017 et récemment mise à jour, renforcit le rôle de l'INESSS dans l'évaluation des innovations à différents moments du cycle de vie. Nos travaux des dernières années ont notamment permis de développer et renforcer nos méthodes d'évaluation, afin qu'elles soient toujours mieux adaptées aux particularités de l'innovation et alignées avec les plus récents développements méthodologiques.

Comment définissez-vous le VBHC et quelle est la pertinence de ce concept pour l'INESSS et le système de santé québécois?

Je vois la valeur comme une meilleure qualité de soins, une meilleure sécurité et une meilleure expérience pour le patient. Un système de soins de santé axé sur la valeur tient également compte du bien-être des prestataires de soins, ce qui se répercute bien entendu sur la qualité des soins offerts aux patients. Il y a aussi la question de l'équité : comment les habitants des régions éloignées vont-ils avoir accès à un nouveau traitement sophistiqué disponible uniquement dans un hôpital tertiaire de Montréal? Dans notre province, c'est une préoccupation constante. À l'INESSS, nous cherchons à intégrer tous ces aspects de la valeur à notre plan stratégique. On pourrait dire que nous avons inscrit la valeur dans notre ADN.

Comment voyez-vous le croisement entre les données probantes en contexte réel (*RWD en anglais*) et le VBHC, aujourd'hui et dans l'avenir?

Le déploiement de la pleine valeur des médicaments exige qu'un usage optimal en soit fait dans notre système de santé – le donner aux bonnes personnes au bon moment – , et le RWD peut soutenir cela. Ces données peuvent nous aider à répondre à des questions telles que : comment les résultats des essais cliniques se traduisent-ils chez les patients dans des conditions réelles de soins, qui peuvent ne pas avoir le même état de santé ou les mêmes comorbidités que les sujets des essais cliniques? Est-ce que nous donnons le médicament aux bonnes personnes? Comment se compare-t-il au standard de soins dans différents groupes de patients? C'est ainsi que nous pouvons préciser la réelle valeur d'un médicament.

Cela signifie-t-il qu'il faut restreindre l'accès à certaines innovations?

Lorsque la valeur d'un médicament est démontrée, nous devons faire tout ce qui est en notre pouvoir pour en faciliter l'accès. Mais lorsqu'un médicament ne démontre pas la valeur escomptée, nous avons besoin de mécanismes pour que l'on cesse de le recommander et de l'utiliser. Il ne sert à rien de donner un médicament qui n'apportera pas de valeur à un patient : il ne fonctionnera pas et personne n'en bénéficiera. De plus, cela prive le patient d'un traitement qui pourrait être plus efficace. C'est pourquoi nous progressons vers l'application d'une approche fondée sur le cycle de vie en matière d'évaluation des technologies de la santé, qui met l'accent sur la valeur au sein de l'entièreté du parcours thérapeutique et qui génère et utilise des données du monde réel.

Que pensez-vous de l'évaluation des médications de précision?

Selon moi, le grand défi consiste à évaluer le domaine de la médecine de précision dans son ensemble. Nous ne parlons plus seulement de médicaments : les thérapies cellulaires par exemple, peuvent prendre la forme de médicaments et avoir un numéro d'identification d'un médicament (DIN), alors qu'il s'agit d'approches beaucoup plus complexes, qui mettent en tension l'organisation des soins et services. Nous avons besoin de nouvelles façons de conceptualiser et d'évaluer ces traitements. L'innovation scientifique est si rapide que nous devons toujours faire du rattrapage. Et c'est ce que nous faisons! Nous devons constamment nous adapter à ce qui vient par la suite, même si nous ne savons pas toujours à quoi cette « suite » ressemblera.

Pouvez-vous nous parler du concept de partenariat avec les patients?

Les traitements médicaux sont devenus de plus en plus complexes, et nous avons besoin d'une expertise plus grande et plus variée pour prendre des décisions éclairées. Les patients ont une expertise unique, car eux seuls peuvent nous dire ce que c'est que de vivre avec un cancer, par exemple, ce qu'ils attendent du traitement et ce qu'ils en retirent. Ils peuvent aussi nous aider à mettre en perspective l'importance des effets secondaires de certains traitements en comparaison avec les bénéfices rapportés.

Comment les patients se sentent-ils à l'idée d'assumer ce rôle accru?

Ils sont très enthousiastes à l'idée, et ils démontrent une compréhension remarquable du concept de valeur. Je crois que le fait d'intégrer des patients et des citoyens dans les processus d'évaluation, et d'être plus transparents dans nos processus permet à ceux-ci de réaliser que l'État ne peut pas prendre tous les risques pour des produits dont les résultats sont incertains. Le risque devrait être partagé. Nous cherchons toujours aussi à informer les patients de manière transparente à l'égard de médicaments dont la valeur est prometteuse, mais incertaine, de sorte que s'ils ont finalement accès à ces médicaments, ils auront eu ce que j'appelle un « accès éclairé ». Les bénéfices à moyen, long terme et les effets secondaires sont parfois très incertains et les patients doivent en être informés et rassurés sur le fait qu'il y a une forme de vigilance qui est de mise au-delà de la décision de remboursement.

Dans quelle mesure le Québec est-il prêt à intégrer le VBHC à tous les niveaux des soins de santé? Quels obstacles pouvez-vous déceler?

Je pense que nous sommes dans une bonne position à l'égard du VBHC, et ce, en partie grâce à l'INESSS. Le terme « valeur » s'est démocratisé, et je sens une volonté politique d'intégrer la notion de valeur dans les décisions concernant notre système de santé. Ce qui manque, c'est l'intégration complète des données : les différents silos ne se parlent pas encore, il est donc encore difficile d'obtenir une image complète du parcours du patient. Nous observons également des lacunes dans la collecte de données sur les résultats rapportés par les patients (*PROMs en anglais*) – une de mes préoccupations depuis mon passage au CHUM – , sur les temps d'attente et sur les soins à domicile.

Comment souhaiteriez-vous que le domaine pharmaceutique spécialisé intègre les pratiques fondées sur la valeur?

La rigueur des données RWD recueillies par l'industrie n'est pas tout à fait à la hauteur de ce qu'elle pourrait être. Idéalement, les données devraient aller au-delà des résultats cliniques et porter également sur l'impact d'un traitement sur le système de santé – par exemple, la nécessité de soins intensifs supplémentaires ou de formation du personnel. J'entrevois également des possibilités d'accroître les évaluations comparatives de données du monde réel, c'est-à-dire l'évaluation d'un nouveau traitement par rapport à la norme de soins. C'est ce qui intéresse les décideurs et les payeurs. La comparaison d'un traitement à un placebo n'a pas une grande portée, car presque toutes les pathologies ont déjà une forme de traitement. Donc au-delà de l'efficacité, a-t-on toute l'information nécessaire sur la valeur ajoutée que confère cette nouvelle intervention ou traitement. Le devis de recherche a-t-il permis de capter ces informations précieuses.

Comment souhaiteriez-vous que l'INESSS utilise les données du monde réel à l'avenir? Y a-t-il des limites à l'utilisation des données du monde réel?

Il y a un besoin évident de RWD pour compléter les essais contrôlés randomisés, mais il doit s'agir de données de haute qualité, et les données des registres peuvent, ou non, répondre à cette norme. À l'heure actuelle, la qualité des données et des preuves du monde réel (RWE en anglais) est très variable. Pour progresser, toutes les parties prenantes doivent appliquer des normes de qualité aux données, afin d'en dériver les preuves scientifiques précises et utilisables.

Parfois, la nature du traitement rend difficile la collecte de données. Si l'on prend l'exemple d'un nouveau médicament pour une maladie rare, il faut parfois des années pour obtenir suffisamment de données permettant d'évaluer la valeur du médicament. Les personnes touchées n'ont pas ces années à attendre, et il faut parfois prendre la décision de leur donner accès au médicament immédiatement malgré la grande incertitude.

À l'échelle mondiale, les ententes fondées sur les résultats ont été utilisées pour garantir l'accès en temps opportun à des médications coûteuses et pour répartir le risque financier de ces traitements. Comment de tels accords s'intègrent-ils avec le VBHC et ont-ils une place dans l'écosystème canadien de la médecine spécialisée?

Ces mécanismes visent à partager les risques associés aux traitements dont la valeur est prometteuse, mais incertaine, afin de ne pas en retarder l'accès aux patients. Il y a une place pour ce type de modalités, mais la configuration optimale n'est pas facile à déterminer. Tous s'entendent pour dire que la charge ne peut pas reposer uniquement sur l'État ou le payeur, et certainement pas sur le patient. Toutefois, comment bien répartir le risque afin que la pleine valeur des traitements se concrétise (le bon patient au bon moment) et que les ajustements puissent être faits lorsque ce n'est pas le cas? Dans leur forme actuelle, les ententes s'accompagnent souvent d'un fardeau clinique et administratif important, et les preuves scientifiques de leur efficacité demeurent relativement limitées. Il est donc essentiel que les différentes parties prenantes travaillent ensemble pour préciser des modalités réalistes et efficaces de gérer ou d'atténuer les risques associés à certaines innovations, tout en s'assurant que des balises claires existent pour soutenir le respect des ententes et l'utilisabilité des données qui y sont collectées. La mouvance vers une approche d'évaluation alignée sur le cycle de vie est certainement un pas dans cette direction.

Quelles orientations et initiatives fondées sur la valeur pouvons-nous attendre de l'INESSS dans les mois et les années à venir? Quelles sont les possibilités qui vous enthousiasment le plus?

Je souhaite certainement renforcer notre capacité à répondre aux préoccupations des patients, des cliniciens et des décideurs et ce en temps opportun. La pandémie a renforcé notre agilité, et nous ne voulons pas perdre cet élan. Je suis particulièrement enthousiaste à l'idée de travailler à la mise en place d'un système de santé apprenant – un système qui s'évalue en permanence et s'adapte aux innovations et aux besoins nouveaux.

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What is a Learning Health System (LHS)?

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