

CHALLENGES OF ASSESSING THE VALUE OF TREATMENTS FOR RARE DISEASES: INSIGHTS FROM INESSS

Canadian Organization for Rare Disorders
Fall Conference

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OUTLINE



How INESSS approaches decision-makers' knowledge needs in the context of rare diseases



A lifecycle approach in HTA may indicate shifting **needs and responsibilities** for regulators, HTA bodies, and the industry

QUÉBEC CONTEXT

- **8,7 million people**
(22,3 % of Canadians)
- **Over 77 %** of the population is **French speaking**
- **Over 60 %** of the population is from **Montreal and its peripheral regions**
- Life expectancy at birth: **82,3 years**
- People 65 years and older: **20 % in 2021**
(26 % expected in 2041)



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RARE DISEASES: AN IMPORTANT PUBLIC HEALTH ISSUE IN QUÉBEC

- **More than 7000 diseases**, including a variety of conditions:
 - Up to 700,000 patients in Québec
 - Some regions are particularly impacted due to founding effects (populations of Charlevoix-Saguenay and the First Nations and Inuit)
- **Current challenges for patients:**
 - Obtaining a timely diagnosis
 - Accessing adapted and multidisciplinary care, effective and innovative treatments, reliable information and psychosocial support
 - Additional challenges for people living far from major centers





How does INESSS approaches
decision-makers' needs in the
context of rare diseases

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ABOUT INESSS



MISSION

Promote clinical excellence and the efficient use of resources in the health and social services sector



VISION

Be the reference to inform decisions and practices



VALUES

Excellence
Independence
Openness
Scientific rigor
Transparency
Integrity
Equity

At the crossroads of science, innovation and decision making, the Institute's productions inform decision making and provide essential benchmarks on best practices

- Autonomous government agency linked to the Minister of Health and Social Services – in Québec, Canada
- A team of 275 persons linked to hundreds of experts

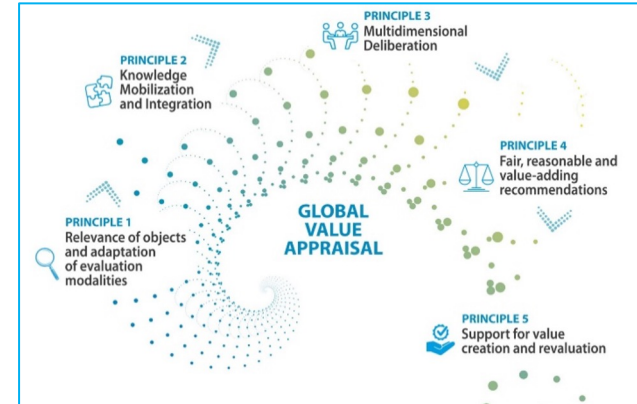
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A GLOBAL VALUE APPRAISAL APPROACH: OUR STATEMENT OF PRINCIPLES AND ETHICAL FOUNDATIONS (ADOPTED IN JULY 2021)

An intervention **provides value** following to the extent to which it:

1. Improves the health and wellbeing of its users (**clinical dimension**);
2. Contributes to a better state of health and well-being for the population as a whole, in alignment with a concern for fairness (**populational dimension**);
3. Optimises the use of resources to support their responsible and sustainable management (**economic dimension**);
4. Fits into the organisational context of care and service delivery in a manner that contributes to strengthen the health and social services system (**organisational dimension**);
5. Fits into the context of the Québec society in a manner that promotes its evolution towards the common good (**sociocultural dimension**).



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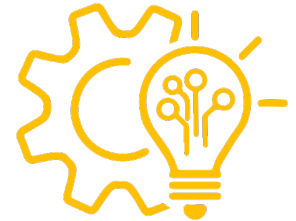
WHY A STATEMENT OF PRINCIPLES AND ETHICAL FOUNDATION?

By stating these principles, INESSS:

Supports **responsible innovation for a sustainable development** of the health and social services system.



*To be sustainable, a health system must adequately deliver across **financial, social and environmental** concerns.*



RESPONSIBLE INNOVATION IN HEALTH (RIH)

WHO

RIH consists of a **collaborative endeavour** wherein innovation stakeholders commit to

WHAT

clarify and meet a set of **ethical, economic, social and environmental** principles, values and requirements

WHEN

when they design, finance, produce, distribute, use and discard sociotechnical solutions

WHY

to address the **needs and challenges of health systems** in a sustainable way.

THE FEDERAL AND PROVINCIAL POLICY CONTEXTS



Goals of the **Canadian** strategy for high-cost drugs for rare diseases (2021):

- Improving patient access and ensuring that it is consistent across the country
- Informing decisions with the best available evidence
- Preserving the sustainability of the health care system



Highlights of the **Québec's** strategy for rare diseases (2022)

AXIS 1: Improved awareness and training (e.g., clinical tools)

AXIS 2: Facilitated and equitable access to diagnosis, care and services (e.g., regional centers)

AXIS 3: Promotion of research, innovation and data collection (e.g., registries, reassessment)

Building a National Strategy for Drugs for Rare Diseases: What We Heard from Canadians Health Canada. July 2021. <https://www.canada.ca/en/health-canada/programs/consultation-national-strategy-high-cost-drugs-rare-diseases-online-engagement/what-we-heard.html>

Politique québécoise pour les maladies rares. Gouvernement du Québec, 2022. <https://publications.msss.gouv.qc.ca/msss/document-003397/>

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THE CHALLENGES OF ASSESSING VALUE FOR RARE DISEASES TREATMENTS

High unmet needs

Limited evidence

Wide spectrum of uncertainties

Expensive developments by industry for a small number of patients

High prices

Decisions about reimbursement under difficult conditions

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APRAISING THE VALUE OF INTERVENTIONS THE SPECIFIC CONTEXT OF RARE DISEASES

- Identify and clarify unmet needs **with** patients and caregivers
- Identify how the proposed new technology addresses the needs expressed by patients whenever possible **with** patients
- In-depth evaluation of the available data leading to an assessment of the therapeutic value of the proposed technology
- *In the specific context of rare diseases : special attention to the promise and plausibility of the proposed technology*
- Adaptation of the economic evaluation to the rare disease context
 - Anticipation of the limits of efficiency assessment
 - At the very least, specify the costs and the budgetary impact
- Openness to iterative evaluation for adjustment according to changing data

Mobilization and integration of knowledge

... gathering information beyond efficacy and safety

- Best possible understanding and classification of uncertainties,
- Impact of the integration of the new technology on the care trajectory and more globally on the delivery of care and services over the entire care trajectory;
- Magnitude of the knowledge transfer challenge and the learning curve inherent in many of these new technologies or approaches;
- Issues related to the optimal and relevant use of these technologies;
- Magnitude of the challenge of changing practices.

Multidimensional deliberation

Transparency in reporting the positions expressed in the deliberative exchanges to reflect the deliberative tensions;

These deliberations should lead to a clear and transparent argument, a series of findings that set the table for recommendations;

Aiming for a global appreciation of the value, reconciling the necessary perspectives, aiming at the common good.

HOW TO SUPPORT SOCIAL SUSTAINABILITY?

TENSION BETWEEN

MAXIMIZING COLLECTIVE WELL-BEING

- The demonstration of modest effectiveness, low level of evidence, with high degree of uncertainty
- Significant costs associated with reimbursement / High per capita costs



ASSISTING THE PERSON IN DIFFICULTY

- The importance of unmet health needs
- The desire to give the population access to potentially beneficial treatments
- Compassion and solidarity require exploring new ways of doing things



Media coverage exacerbates this tension and underplays the need to offer effective and safe interventions.

VALUE APPRAISAL AT AN EARLY STAGE AND BEYOND

Responsible Innovation in Health (RIH) was developed to steer, **at an early stage**, innovation towards equitable as well as economically and environmentally sustainable health systems

It offers food for thought for stakeholders in the field of rare diseases



There is a need to:

- Pay attention to the **direction** innovation takes — the future in which it engages all parties — and the **plausibility** of value creation
- Gather evidence beyond efficacy and safety AND beyond early conditional reimbursement decisions
- To ensure that fair and reasonable HTA recommendations resist the test of time and lead to disinvestment when appropriate

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WHAT ARE THE PRACTICAL CONSIDERATIONS IN USING A LIFECYCLE APPROACH?

Evolve the evidence



- A real-world evidence (RWE) approach cannot replace methodologically sound RCTs, when they are feasible
- Multicenter and/or pragmatic trials could be funded through dedicated programs
- “Greater sharing of clinical data and HTA learnings” is recommended (Trowman et al., 2023)
- The scope, frequency and burden or (re)assessments require careful thinking

Manage the uncertainties



- RWE may not reduce the decisional uncertainties that matter the most
- Regulators’ “decisions are bounded by the mission of their agencies, which is to decide whether market access is warranted or not” (Lehoux et al., 2017)
- Need to assess the performance of managed entry agreements to ensure that those “administratively burdensome and ineffective” are improved or withdrawn (Trowman et al., 2023)

CONCLUSION

- “Responsible innovation means **taking care of the future** through collective stewardship of science and innovation in the present” (Stligoe et al., 2013)
- Value-based decisions in rare diseases require **clearly defined milestones** as well as **obligations** for all parties involved
 - Need to anticipate and mitigate both short- and long-term uncertainties
 - And to remain true to our respective missions
- While INESSS keeps evolving its methods and adapting to a complex context:

→ Issues surrounding reimbursement under difficult conditions must be made explicit, deliberated, and aligned with societal priorities

→ INESSS will rely on this social consensus to formulate fair, reasonable and responsible recommendations



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Thank you for your attention!

Merci!



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