A methodology for the evaluation of a disruptive innovative therapy: The example of Kymriah®


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BACKGROUND
Establishing the efficacy and cost-effectiveness of innovative health technologies poses important challenges due to the uncertainty and paucity of evidence. Herein, we describe strategies used to address these challenges in our assessment of an immunocellular therapy, Kymriah®, that has been proposed for the treatment of advanced forms of diffuse large B cell lymphoma (DLBCL) and lymphoblastic leukemia (ALL).

EVALUATION OF INNOVATIVE TECHNOLOGIES AT INESSS

- Early dialogue between relevant stakeholders
- Evaluation of the promise
- Evaluation criteria
- Evaluation of real benefits and optimal use
- Development of contextualized evidence
- Capture of implementation challenges

RESULTS

DEcision-Making Need
What are the therapeutic value and the cost-effectiveness of Kymriah® and what are the organizational issues relating to its adoption in Quebec?

Scientific Data
Systematic literature review:
- Primary studies
- HTA reports
- Grey literature

Economic analysis:
- Cost-effectiveness analysis
- Budget impact analysis

Contextual Data
- Consultation with clinical expert committee
- Consultation with health services expert committee
- Use of medico-administrative databases

EXPERiential Data
- Consultation with clinical & hospital management teams
- Interview of patients or patient representatives
- Consultation with citizens

RESULT OF THE ASSESSMENT OF KYMRIAH®

Key findings
- Promising therapy associated with high level of toxicity
- Immature body of evidence (short follow-up)
- Methodological limitations (indirect comparison)
- Uncertainty of long-term clinical results & cost-effectiveness

Concerns
- Significant impact on hospital management
- Burden of demonstrating the value of Kymriah®
- Unfair access to the treatment
- Solidifying Quebec’s expertise in cellular therapy

Indication
For the treatment of pediatric and young adult patients aged 3 to 25 years with ALL who:
- are refractory or
- have relapsed after an allogenic stem cell transplant or
- for those who are ineligible for a transplant or have experienced second or later relapse.

Recom mendati on
Coverage of Kymriah® in:

B-ALL
- mitigation of the economic burden
- temporary status for such coverage (3 years)
- development of evidence

DBLCL
- mitigation of the economic burden

Implementation considerations
1. Careful planning of service offer so as not to compromise access to routine care.
2. Real-world data collection by the manufacturer

CONCLUSION

A coverage decision conditional on limited access and collection of real-world data may be an appropriate policy choice to address uncertainties related to promising new technologies. It allows patients early access to innovative therapies while ensuring careful monitoring of long-term patient risks and of the cost burden to the healthcare system.

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Disclosures
We have not had an affiliation (financial or otherwise) with a commercial organization that may have a direct or indirect connection with the content of this presentation.

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