

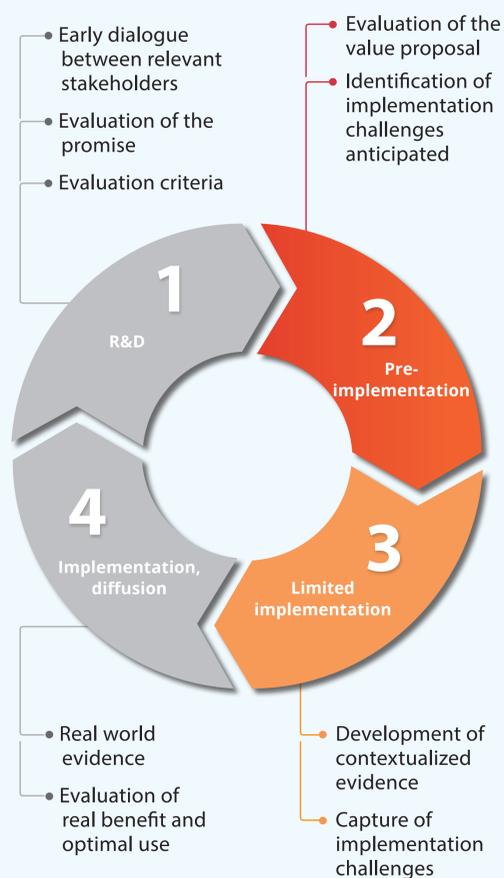
# A methodology for the evaluation of a disruptive innovative therapy: The example of Kymriah®

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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 **acute lymphoblastic leukemia (ALL)** and **diffuse large B cell lymphoma (DLBCL)**.

## EVALUATION OF INNOVATIVE TECHNOLOGIES AT INESSS



## RESULTS

### DECISION-MAKING NEED

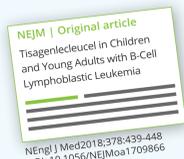


What are the **therapeutic value** and the **cost-effectiveness** of Kymriah® and what are the organizational issues relating to its adoption in Quebec?

### 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

SCIENTIFIC DATA

DATA SYNTHESIS

CONTEXTUAL DATA

EXPERIENTIAL DATA

### 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

#### RECOMMENDATION Coverage of Kymriah® if:

B-ALL	DLBCL
<ul style="list-style-type: none"> <li>▶ mitigation of the economic burden</li> </ul>	<ul style="list-style-type: none"> <li>▶ mitigation of the economic burden</li> <li>▶ temporary status for such coverage (3 years)</li> <li>▶ development of evidence</li> </ul>
<p><b>Indication</b> For the treatment of pediatric and young adult patients aged 3 to 25 years with <b>ALL</b> who:</p> <ul style="list-style-type: none"> <li>▶ are refractory <b>or</b></li> <li>▶ have relapsed after an allogeneic stem cell transplant <b>or</b></li> <li>▶ for those who are ineligible for a transplant or have experienced second or later relapse.</li> </ul>	<p><b>Indication</b> For the treatment of adult patients with relapsed or refractory <b>DLBCL</b> not otherwise specified, high grade B-cell lymphoma and <b>DLBCL</b> arising from follicular lymphoma.</p>

#### 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.

### Acknowledgements

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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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### REFERENCES

- Maude SL et al. Tisagenlecleucel in children and young adults with B-cell lymphoblastic leukemia. *N Engl J Med* 2018, Feb 1; 378:439-448.
- Schuster JS et al. Tisagenlecleucel in adult relapsed or refractory diffuse large B-cell lymphoma. *N Engl J Med* 2019, Jan 3; 380:45-56.
- Tisagenlecleucel pour le traitement de la leucémie lymphoblastique aiguë à cellules B récidivante ou réfractaire. Rapport rédigé par Bisailon R, Mombo N N, Beha S, Brouard M-E, Arbour S et Béland M. Québec, Qc : INESSS; 72 p.
- Tisagenlecleucel pour le traitement du lymphome difus à grandes cellules B récidivant ou réfractaire. Rapport rédigé par Mombo N N, Bisailon R, Beha S, Brouard M-E, Arbour S et Béland M. Québec, Qc : INESSS; 67 p.