Poster #EE266

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Perez-Kempner, Lucia

Budhia, Sangeeta

The economics promises of a cure - Best practices to guide economic negotiations for the reimbursement of curative therapies for cancer

Background

Despite the advances observed in the oncology field in the last 30 years, most oncology drugs are not able to offer a cure. Nonetheless, recent cell therapies have been launched with the promises of a curative value proposition. While each therapy needs to provide clinical evidence to substantiate this, the clinical evidence presented also needs to translate into an economic value attribute. For therapies with a curative value proposition, we analyzed the economic evaluations presented in health technology assessment (HTA) reports and identified critiques that challenged the economic value of the new therapies in the healthcare system.

Methods

CAR-T drugs were selected given their curative claims, novel technology, and early data evaluations. HTA agencies in countries with an economic evaluation component were selected, namely Australia (PBAC), Canada (CADTH), England (NICE), and Sweden (TLV), to analyze the global HTA perspective and review HTA reports for CAR-T drugs in diseases where these have been launched and have been assessed by HTA agencies (i.e., relapsed/refractory (R/R) diffuse large B-cell lymphoma (DLBCL), R/R follicular lymphoma (FL), and R/R mantel cell lymphoma (MCL)). Each report was qualitatively analyzed to identify critiques raised by HTA agencies on the presented economic evaluations.

REFERENCES

HTA appraisals reports issued by NICE for Tisagenlecleucel and Axicabtagene ciloleucel in R/R DLBCL and R/R FL and for Brexucabtagene autoleucel in R/R MCL; HTA appraisals reports issued by TLV for Tisagenlecleucel and Axicabtagene ciloleucel in R/R DLBCL and for Brexucabtagene autoleucel in R/R MCL; HTA appraisals reports issued by CADTH for Tisagenlecleucel and Axicabtagene ciloleucel in R/R DLBCL and R/R FL and for Brexucabtagene autoleucel in R/R MCL; HTA appraisals reports issued by MSAC for Tisagenlecleucel in R/R DLBCL, for Axicabtagene ciloleucel in R/R DLBCL and R/R FL and for Brexucabtagene autoleucel in R/R MCL.

Results

We identified 16 HTA reports across all agencies (**Table 1**). HTA agencies preferred 3-health state (progression-free; progressed; death) models for economic evaluations, with a time horizon reflective of the natural history of the disease (**Figure 1**). Due to clinical uncertainties to support curative claims (e.g., short follow-up time, patient size), HTA agencies mostly accepted conservative approaches to curative value propositions, such as a short time horizon, a short cure rate, and a higher mortality risk for the treated population compared to the overall population over a lifetime (**Figure 1**). Moreover, in the absence of mature survival data, HTA agencies preferred survival values modelled through spline/standard-parametric rather than mixture-cure models, requesting survival assumptions to be in line with the data collected through clinical trials (**Figure 1**). Finally, HTA agencies preferred utility values derived from clinical trials and challenged drug cost inputs different from the costs of reimbursed therapies (i.e., costs sourced from the literature) (**Figure 1**).

Conclusions

The economic value of curative drugs is constrained by clinical trial limitations that pose uncertainties on curative claims and economic evaluations. So far, HTA agencies prefer conservative economic evaluations that fit the clinical data and local context rather than innovative models that assume a cure that may not yet be clinically demonstrated. Early local health economics, the acceptance of innovative models by HTA agencies that better demonstrate the curative value for patients, faster and more informative clinical and real-world evidence studies, and payer consultations are critical to ensure the development of robust economic evaluations.

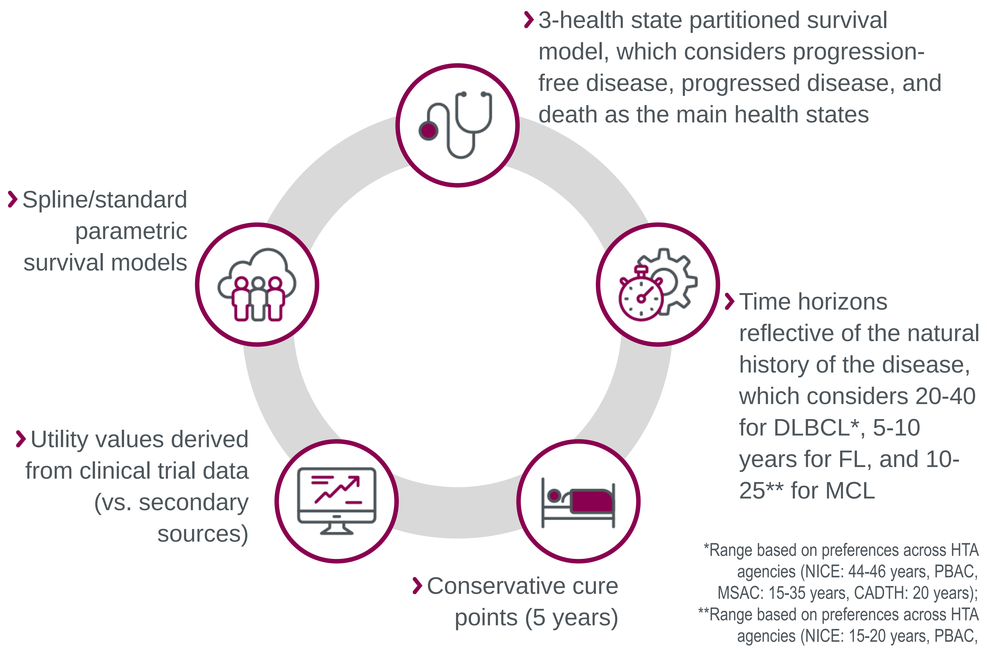
Table 1. List of selected CAR-T drugs across the three oncology indications

	Tisagenlecleucel		Axicabtagene ciloleucel		Brexucabtagene autoleucel
	R/R DLBCL	R/R FL	R/R DLBCL	R/R FL	R/R MCL
NICE Decision	Recommended (CDF) (2019)	Terminated	Recommended (CDF) (2019)	N/A*	Recommended (2022)
TLV Decision	Not recommended (2019)	N/A*	Recommended (2019)	N/A*	No decision**
CADTH Decision	Recommended (2019)	Recommended (2023)	Recommended (2019)	Recommended (2023)	Recommended (2021)
MSAC Decision	Recommended (2019)[6]	N/A*	Recommended (2020)[6]	Recommended (2019)	Recommended (2021)

CADTH: Canadian Agency for Drugs and Technologies in Health; MSAC: Medical Services Advisory Committee; NICE: National Institute for Health and Care Excellence; TLV:Tandvårds-Läkemedelförmånsverket.

*N/A: Not Available: HTA report not available; **No decision made by TLV: TLV produced health economic assessment of the data as per request of the NT council. The NT Council and the regions make their recommendations independently based on TLV's health economic assessment among other things.

Figure 1. Preferred recommendations for the economic modelling of CAR-T drugs, as per the HTA reports identified



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