Utilization and Impact of Value Assessment Frameworks on Payer Decisions Regarding Chimeric Antigen Receptor T-cell Therapies

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BACKGROUND

- Over the past several years, value assessment frameworks (VAFs) have become of interest to payers and other stakeholders and many of them are specifically designed for evaluation of oncology therapies. The frameworks of interest were developed by the American Society of Clinical Oncology (ASCO), the European Society for Medical Oncology (ESMO), the Institute for Clinical and Economic Review (ICER), the National Comprehensive Cancer Network® (NCCN), Memorial Sloan Kettering Cancer Center (MSKCC), and a collaboration between Avalere® Health and FasterCures.[1-6]
- The primary goal of these VAFs is to provide a standard methodology for generating evidence to assist decision makers and other stakeholders in determining which therapies provide the most benefit for patients and maximize allocation of healthcare resources.[7,8]
- Despite the increasingly broad awareness of VAFs, recent therapeutic advances in oncology introduce new, if not unique, considerations and challenges in valuing novel cancer therapies and in the application of VAFs by payers in their formulary decision-making.
- Specifically, chimeric antigen receptor T-cell (CAR-T) therapies represent a novel type of cancer treatment in which a patient's own T-cells are genetically engineered with receptors that signal and bind with surface proteins on malignant cells leading to antitumor activity.[9]
  - The Federal Food and Drug Administration (FDA) approved 2 CAR-T therapies, KYMRIAH™ (tisagenlecleucel) and YESCARTA® (axicabtagene ciloleucel) in 2017.
  - Subsequently, ICER released an evidence report evaluating these CAR-T therapies in March 2018.
- To date, there has been limited evidence or discussion regarding payer perception and utilization of VAFs, particularly with respect to CAR-T therapies.

OBJECTIVES

- This study aimed to evaluate United States (US) payer perceptions associated with the current and future utilizations of VAFs for cancer in general, and specifically the application of VAFs in decision-making for CAR-T therapies.

METHODS

Study Design

- An electronic survey was developed to query US pharmacy and medical directors from managed care organizations about their familiarity and utilization of VAFs.
- All respondents were required to have ≥1 year experience at their current managed care organization and have served or be currently serving as active members of their organization's pharmacy and therapeutics (P&T) committees.
- Respondents were excluded if they had <1 year experience, were part of a consulting organization, or have not served on a P&T committee.
- Participation in this survey was voluntary and a modest honorarium was paid by Xcenda to participants who completed the survey.

Survey Design and Implementation

- The survey assessed payers' familiarity with VAFs used for oncology and their application in formulary reviews of cancer in general, and specifically for CAR-T therapies.
- VAFs considered in the survey included ASCO Net Health Benefit (NHB), ESMO Magnitude of Clinical Benefit Scale (MCBS), ICER evidence reports, MSKCC DrugAbacus (currently DrugPricing Lab), NCCN Evidence Blocks™, and Avalere® Health/FasterCures Patient Perspective Value Framework (PPVF).
- The survey was fielded to 114 advisors within Xcenda's Managed Care Network (MCN) during December 2017.
  - Survey included 16 questions that:
    - Evaluted payer perception and utilization of VAFs for formulary decisions regarding cancer treatments in general and CAR-T therapies.
    - Queried any direct involvement they had with ICER during development of CAR-T evidence reports (Draft report: December 18, 2017; final report: March 28, 2018).
  - Survey questions were posed as dichotomous yes or no, multiple choice, and 7-point Likert scale ratings (1=Not at all familiar/impactful and 7=extremely familiar/impactful).

RESULTS

Participation Rate and Demographics

- Overall, 60 (52.6%) survey responses were completed, of which 59 (51.8%) payers met eligibility requirements (**Table 1**).
  - One respondent was excluded due to the absence of P&T committee experience.

Table 1. Payer Demographic Characteristics (N=59)

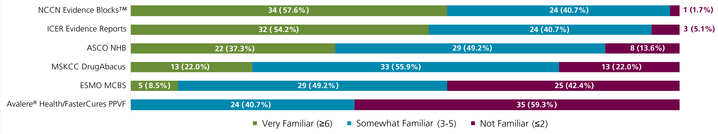
Demographics		n (%)
Payer Role	Medical director	14 (24)
	Pharmacy director	40 (68)
	Other	4 (8)
Lives Covered	≤500,000	21 (36)
	500,001–2,500,000	18 (30)
	>2,500,000	20 (34)
Years of Experience	1–5	15 (25)
	6–10	16 (27)
	11–15	13 (22)
	16–20	7 (12)
	20+	8 (14)
Geographic Coverage	National	17 (29)
	Regional	42 (71)

Payer Familiarity With the VAFs (Figure 1)

- Almost all payers (≥95%) were at least "moderately familiar" with all of the VAFs and over half of responders were "very familiar" with NCCN Evidence Blocks™ and ICER evidence reports.
- Approximately one-third of payers reported they were "very familiar" with the ASCO NHB, but less than one-quarter of respondents were "very familiar" with the MSKCC DrugAbacus and ESMO MSCB.
- Almost two-thirds of respondents reported having very little familiarity with the PPVF which focuses specifically on the aspects of value most important to patients.

RESULTS (cont.)

Figure 1. Payer Familiarity With the VAFs (N=59)



Question: On a scale of 1–7, rate level of familiarity with each VAF (N=59)
Key: ASCO NHB – American Society of Clinical Oncology Net Health Benefit; ESMO MCBS – European Society for Medical Oncology Magnitude of Clinical Benefit Scale; ICER – Institute for Clinical and Economic Review; MSKCC – Memorial Sloan Kettering Cancer Center; NCCN – National Comprehensive Cancer Network®; PPVF – Patient Perspective Value Framework.

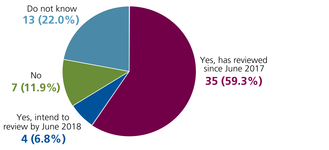
Reported Coverage Evaluation of CAR-T Therapies

- Thirty-five respondents reported reviewing coverage of CAR-T therapies since June 1, 2017 and 4 respondents anticipated reviewing it before June 1, 2018 (**Figure 2**).
- One-third of respondents were unsure or had no plans to review CAR-T coverage prior to June 2018.

VAF Utilization by P&T Committees for Cancer in General and CAR-T Therapies

- The most commonly referenced VAFs for cancer in general (N=59) and CAR-T therapies (N=35) were the NCCN Evidence Blocks™, ICER evidence reports, and ASCO NHB (**Figure 3**).

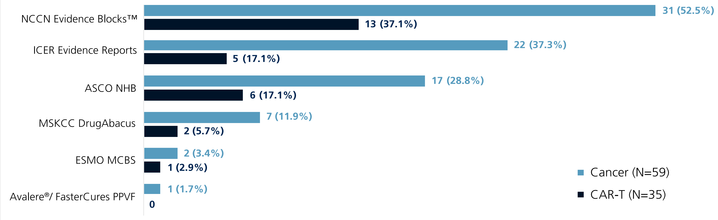
Figure 2. P&T Committee Reviews of CAR-T Therapies for Coverage Decisions (N=59)



Question: Has your organization reviewed CAR-T therapies since June 1, 2017 or intends to by June 1, 2018? (N=59)
Key: CAR-T – chimeric receptor antigen T-cell; P&T – pharmacy and therapeutics.

- Among all 59 payers that reviewed any cancer therapies, NCCN Evidence Blocks™ were used by 31 (52.5%) payers, while a majority of payers did not utilize other VAFs.
- Among the 35 payers that reviewed CAR-T therapies, a majority did not utilize VAFs.
  - Leading payer reasons for not using VAFs in CAR-T therapies coverage decisions included lack of added value, no/minimal experience with VAFs, and validity concerns across all VAFs.

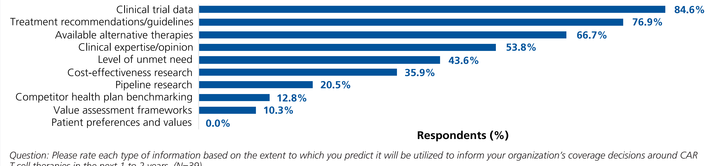
Figure 3. VAF Utilization for Cancer Therapies in General vs CAR-T Therapies[a]



Has your organization used any of the listed value assessment framework(s) as part of your formulary reviews for (cancer/CAR-T) therapies since (January 1, 2015/June 1, 2017)?
[a] Participants had the option to answer "Do not know," which is not included in this graph.
Key: ASCO NHB – American Society of Clinical Oncology Net Health Benefit; CAR-T – chimeric receptor antigen T-cell; ESMO MCBS – European Society for Medical Oncology Magnitude of Clinical Benefit Scale; ICER – Institute for Clinical and Economic Review; MSKCC – Memorial Sloan Kettering Cancer Center; NCCN – National Comprehensive Cancer Network®; PPVF – Patient Perspective Value Framework.

- Among respondents who have recently reviewed or are anticipating review of CAR-T therapies (N=39), a majority (n=33; 84.6%) indicated that the most important information to have for future coverage decisions were clinical trial data, followed by treatment recommendations/guidelines (n=30; 76.9%), and available alternative therapies (n=26; 66.7%) (**Figure 4**).
- Conversely, respondents reported little to no need for information on patient preference and values (n=0; 0%), value assessment frameworks (n=4; 10.3%), and competitor health plan benchmarking (n=5; 12.8%).

Figure 4. Information to Improve CAR-T Therapies Coverage Decisions (N=39)

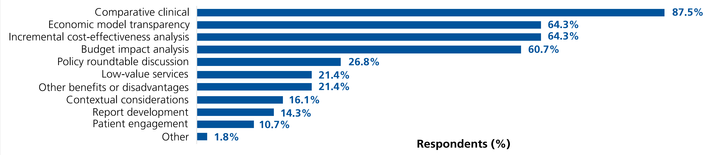


Question: Please rate each type of information based on the extent to which you predict it will be utilized to inform your organization's coverage decisions around CAR T-cell therapies in the next 1 to 2 years. (N=39)
Key: CAR-T – chimeric antigen receptor T-cell.

Engagement and Utilization of ICER Evidence Reports

- The majority of respondents (n=31; 52.5%) have not, and were not planning to engage with ICER on any evaluation topics.
  - Only 2 (3.3%) respondents noted that their organization had engaged with ICER in the evaluation of CAR-T therapies.
  - In contrast, 22 (37.3%) respondents responded they had not yet engaged with ICER, but anticipated future engagement as a stakeholder.
- Approximately two-thirds of payers (67.8%) reported that they expected the ICER CAR-T evidence report to have at least a modest impact (≥4 on 7-point Likert scale) on CAR-T therapies coverage.
  - A majority of payers identified comparative clinical and several economic sections of ICER's CAR-T evidence report to be most valuable for informing coverage decisions (**Figure 5**).

Figure 5. Valuing ICER Evidence Report Components (N=56)[a]



Question: Which of the following ICER report components do you believe will be most valuable to your organization's coverage decision around CAR T-cell therapies? (N=56)
[a] 3 respondents were excluded for ranking ICER evidence report impact of 0 or 1.
Key: CAR-T – chimeric receptor antigen T-cell; ICER – Institute for Clinical and Economic Review.

LIMITATIONS

- This study considered only a predetermined group of VAFs within the survey and therefore did not account for all potential VAFs that may be utilized in coverage decision making. These VAFs were selected based on relevance to oncology therapies and their recent visibility in the healthcare field. Nevertheless, some P&T committees may have internal assessment tools developed by their health plans for value-based decision making which have not been considered here.
- Rationale for not utilizing specific VAFs in general oncology decision making and potential aspects of evidence that would help better inform CART-T therapies decision making were included as pre-specified lists of items that payers were asked to rate and rank in order of relative importance. Therefore, there may be additional aspects that are also relevant to decision-making that are not captured in our survey.
- This survey was conducted prior to the release of ICER's CAR-T evidence report and the corresponding public meeting. As such, our results reflect the payers' perspectives, perceived and anticipated utilization of ICER's evidence report at this interim time in the evaluation process and may not have captured utilization or engagement in the process occurring after the survey.
- Generalizing the results of our study should be done with caution, as the responses reflect the perspectives of a select group of respondents representing health plan P&T committees and are derived from a relatively limited sample size.

CONCLUSION

- Payers representing P&T committees are restrained in their uptake and utilization of VAFs for coverage decision making in oncology. While they acknowledge that VAFs can provide useful background information, respondents did not report substantial use or consideration of VAFs as a factor in coverage decisions for cancer therapies in general or CAR-T therapies.
- With the growing focus on value-driven decision making, there remains a considerable opportunity to improve the understanding and use of VAFs and facilitate timely assessment and access to CAR-T therapies, as well as future cancer therapies.
- There is a future opportunity to resurvey payers representing P&T committees to confirm that their value of clinical benefit is consistent with the ICER CAR-T evidence report, probe any concerns on affordability/access, and explore additional policy recommendations.

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