Tabelecleucel for Epstein-Barr Virus Post-Transplant Lymphoproliferative Disease: Effectiveness and Value

Public Meeting — November 14, 2024

Meeting materials available at: https://icer.org/assessment/ebv-ptld-2024/

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NEW ENGLAND CEPAC
COMPARATIVE EFFECTIVENESS PUBLIC ADVISORY COUNCIL

Patient Experts

Joseph A. Kopec, Patient

- *No conflicts to disclose.*

Doug Worthen, Patient

- *No conflicts to disclose.*

Clinical Experts

- **Upton Allen, O.Ont., CD, MBBS, MSc, FAAP, FRCPC, Hon FRCP, FIDSA,** Chief, Division of Infectious Disease, The Hospital for Sick Children; Senior Associate Scientist, Research Institute, The Hospital for Sick Children; Professor, Pediatrics, University of Toronto
- *No conflicts to disclose.*

Sarah Nikiforow, MD, PhD, Technical Director, Immune Effector Cell Therapy Program, Dana-Farber Cancer Institute

- *Dr. Sarah Nikiforow served as a PI at Dana-Farber Cancer Center for Tabelecleucel on Atara Biotherapeutics studies (CTL 201, CTL 901, CTL 302, CTL 301, CTL 205), but she did not accept any salary support or payment for serving as PI.*

ICER Speakers

Sarah K. Emond, MPP
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Grace Lin, MD
Evidence Author

Marina Richardson, PhD, MSc
Economic Modeler

Steven D. Pearson, MD, MSc
Special Advisor

Why are we here today?

“It was a really complicated and frustrating time…I was wanting to be healthy again…from all of the different treatments and everything [after transplant]. And so, they thought that I had gut GVHD…so they were giving me immunosuppressants…because of the immunosuppressants, I think that’s when the EBV+ PTLD went nuts throughout my GI track. I went back into the hospital and was 120 pounds…”

“…My dad…he pretty much took time off work to become an expert in this…to be my main patient advocate. My mom moved [to locations where the patient needed treatment]….to have a place where I could stay outpatient…it was really hard on the family. How do you say no to what you think is the best care? Its like, you kind of try to just make it work.”

Patient living with EBV+ PTLD

Why Are We Here Today?

- What happens the day these treatments receive FDA approval?
- Questions about:
  - What are the risks and benefits?
  - How do new treatments fit into the evolving landscape?
  - What are reasonable prices and costs to patients, the health system, and the government?
  - What lessons are being learned to guide our actions in the future?

The Impact on Rising Health Care Costs for Everyone

DIAGNOSIS: DEBT

100 Million People in America Are Saddled With Health Care Debt

By Noam N. Levey

JUNE 16, 2022

Why Delaware is eying a 27% premium hike on state employees' health insurance

Amanda Fries

Delaware News Journal

Published 4:35 a.m. ET Feb. 1, 2024 | **Updated 9:29 p.m. ET Feb. 6, 2024**

100 Million People in America Are Saddled With Health Care Debt (KFF Health News)

Why Delaware is eying a 27% premium hike on state employees' health insurance (Delaware Online)

Organizational Overview

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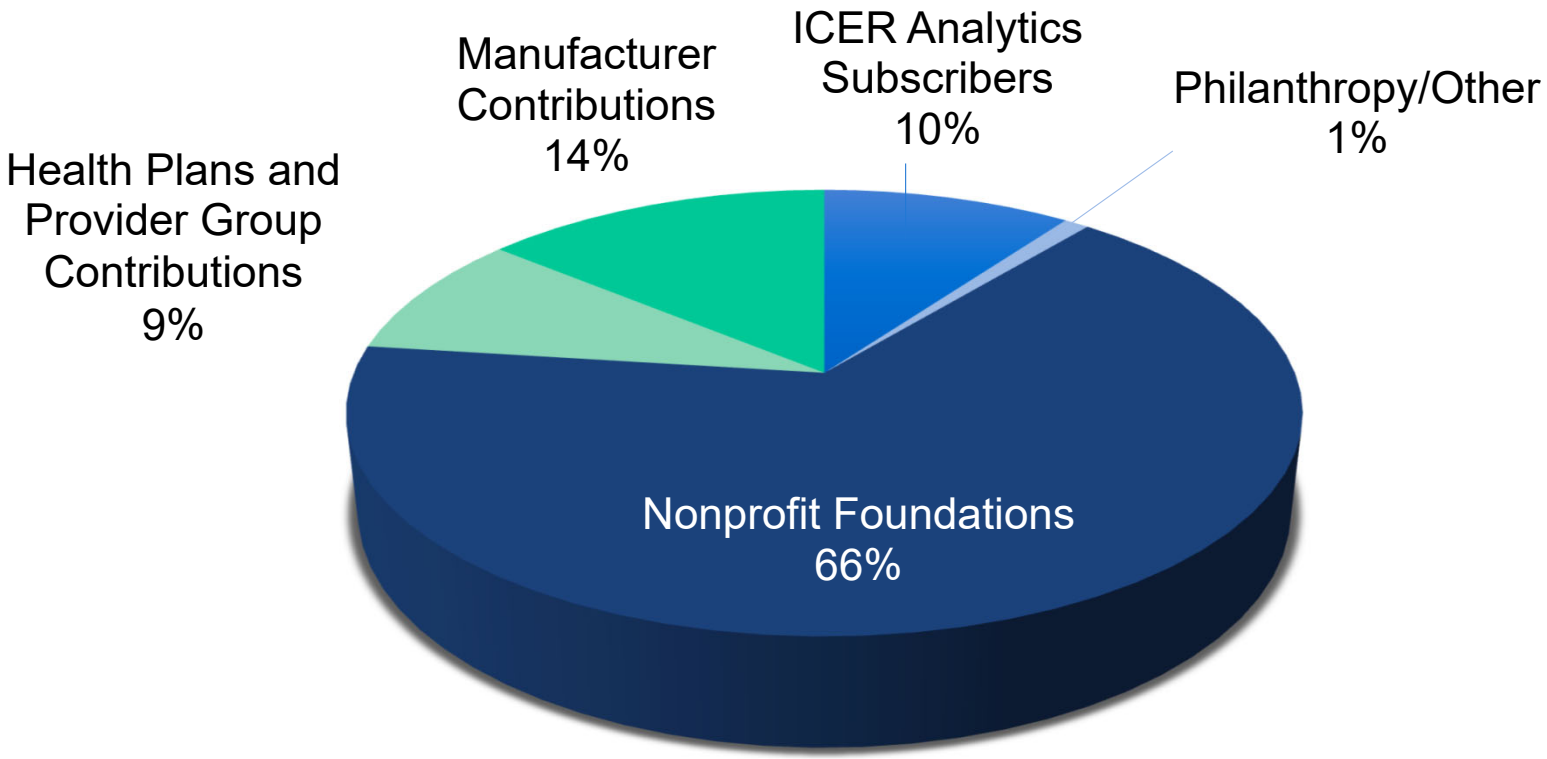
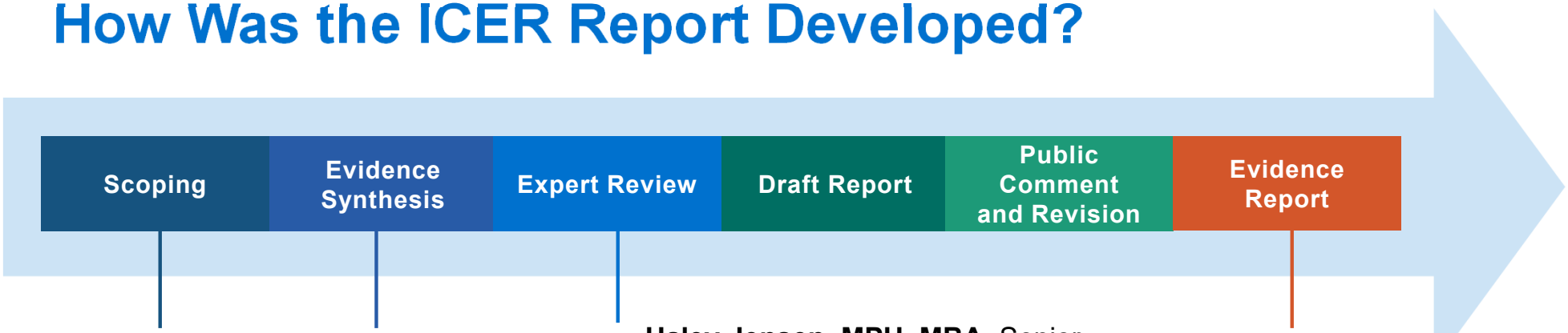
Funding 2024



*ICER received significant funding from Arnold Ventures, The California Health Care Foundation, and The Commonwealth Fund, The Patrick and Catherine Weldon Donaghue Medical Research Foundation, and Peterson Center on Healthcare, LLC. Source: https://icer.org/who-we-are/independentfunding/sources-of-funding/

How Was the ICER Report Developed?



Guidance from patients, clinical experts, manufacturers, and other stakeholders

Evidence analysis in collaboration with the **University of California, San Francisco**

- **Haley Jensen, MPH, MBA**, Senior Director, Transplant Programs, National Kidney Foundation
- **Daniel Malone, PhD**, Research Professor, University of Utah
- **Miguel-Angel Perales, MD**, Chief, Adult BMT Services, Memorial Sloan Kettering Cancer Center
- **Susan Prockop, MD,** Associate Attending, Pediatrics, Boston Children's Hospital

Structured to support **New England CEPAC** voting and policy discussion

Value Assessment Framework: Long-Term Value for Money

Agenda (ET)

10:00 AM	Meeting Convened and Opening Remarks
10:20 AM	Presentation of the Clinical Evidence
11:00 AM	Presentation of the Economic Model
11:40 AM	Public Comments and Discussion
12:00 PM	Lunch Break
12:50 PM	NE CEPAC Deliberation and Vote
1:50 PM	Break
2:00 PM	Policy Roundtable Discussion
3:30 PM	Reflections from NE CEPAC
4:00 PM	Meeting Adjourned

Presentation of the Clinical Evidence

Grace Lin, MD, MAS

Medical Director for Health Technology Assessment, ICER

Professor of Medicine and Health Policy, UCSF

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Key Collaborators

Team Role	Assigned Team Member
Research Lead	Avery McKenna, BS
Research Assistant	Finn Raymond, BS

Disclosures

Financial support provided to Grace Lin from the Institute for Clinical and Economic Review (ICER)

Grace Lin, Avery McKenna, and Finn Raymond have no conflicts to disclose defined as more than $10,000 in healthcare company stock or more than $5,000 in honoraria or consultancies relevant to this report during the previous year from health care manufacturers or insurers.

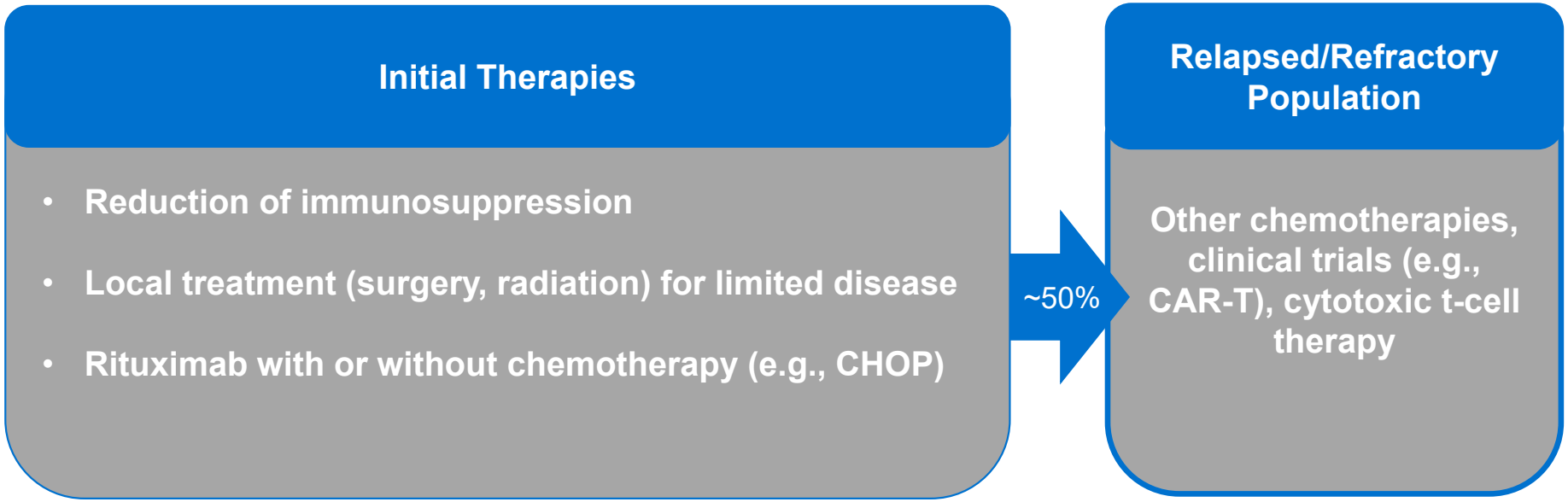
Epstein-Barr Virus Positive Post-Transplant Lymphoproliferative Disease (EBV+ PTLD)

- PTLD is a rare, serious, and often fatal complication of solid organ transplant (SOT) or allogeneic hematopoietic stem cell transplant (HSCT)
- Majority associated with acquisition or reactivation of EBV (EBV+ PTLD)
- Incidence varies based on transplant type
  - <1% to 30% for SOT; 3% for HSCT
  - Risk factors: EBV-negative recipient, transplants requiring higher levels of immunosuppression, age, severe graft-versus-host disease (GvHD)

EBV+ PTLD Symptoms

- People may present with or without symptoms
- Generalized symptoms: fatigue, swollen glands, night sweats, unintended weight loss
- Organ-specific symptoms: most commonly gastrointestinal, lung, or central nervous system
- Majority of cases are monomorphic diffuse B-cell lymphoma subtype

EBV+ PTLD: Treatment Options



Clinical Course

- EBV+ PTLD can be rapidly fatal (weeks to months)
- Survival estimates of 40-60% at 5 years if response to first-line therapy

CHOP: cyclophosphamide, doxorubicin hydrochloride (hydroxydaunorubicin), vincristine sulfate (Oncovin), and prednisone, EBV+ PTLD: Epstein-Barr virus+ post-transplant lymphoproliferative disease

Tabelecleucel (tab-cel®, Ebvallo®)

Off-the-shelf, allogeneic, T-cell immunotherapy

Targets and eliminates EBV-infected cells

EBV-specific T-cells derived from healthy donors based on shared human leukocyte antigens (HLA) restriction and partially matched HLA profile

Administered IV for three doses per 35-day cycle for a minimum of two cycles

EBV: Epstein-Barr virus

Insight from Discussions with Patients

Tremendous impact on physical, social, and emotional functioning of patients and caregivers

Lack of treatment options for relapsed/refractory disease; avoidance of chemotherapy desirable

Difficulty accessing newer therapies: insurance barriers, potential high cost of new treatments

Scope of Review

Population	People with relapsed/refractory EBV+ PTLD who have received at least one prior therapy
Intervention	Tabelecleucel
Comparator	Usual Care, which may include pharmacologic or nonpharmacologic treatment options
Outcomes	• Overall Survival • Objective Response Rate • Safety and Adverse Events

Clinical Evidence

Clinical Trials of Tabelecleucel and Usual Care

Study	N	Study Design	Population
Pivotal Trial of Tabelecleucel			
ALLELE	43	Phase III single-arm open label study	SOT or HSCT recipients with R/R EBV+ PTLD after rituximab ± chemotherapy
Expanded Access Programs (EAP)			
US & EU EAPs	98	Single-arm & Individual Patient EAPs	R/R EBV+ PTLD with no alternative therapeutic options / not eligible for clinical trial enrollment
Usual Care			
Dharnidharka 2021	86	Retrospective chart review	R/R SOT recipients
Socie 2024	81	Retrospective chart review	R/R HSCT recipients
Barlev 2024	114	Comparative analysis of a subset of ALLELE participants (n=30) and a retrospective chart review (n=84)	Same as ALLELE trial above

EAP: expanded access program, EBV+: Epstein-Barr virus positive, EU: European, HSCT: hematopoietic stem cell transplant, PTLD: post-transplant lymphoproliferative disease, R/R: relapsed/refractory, SOT: solid organ transplant, U.S.: United States

Pivotal Trial: ALLELE

Study Design

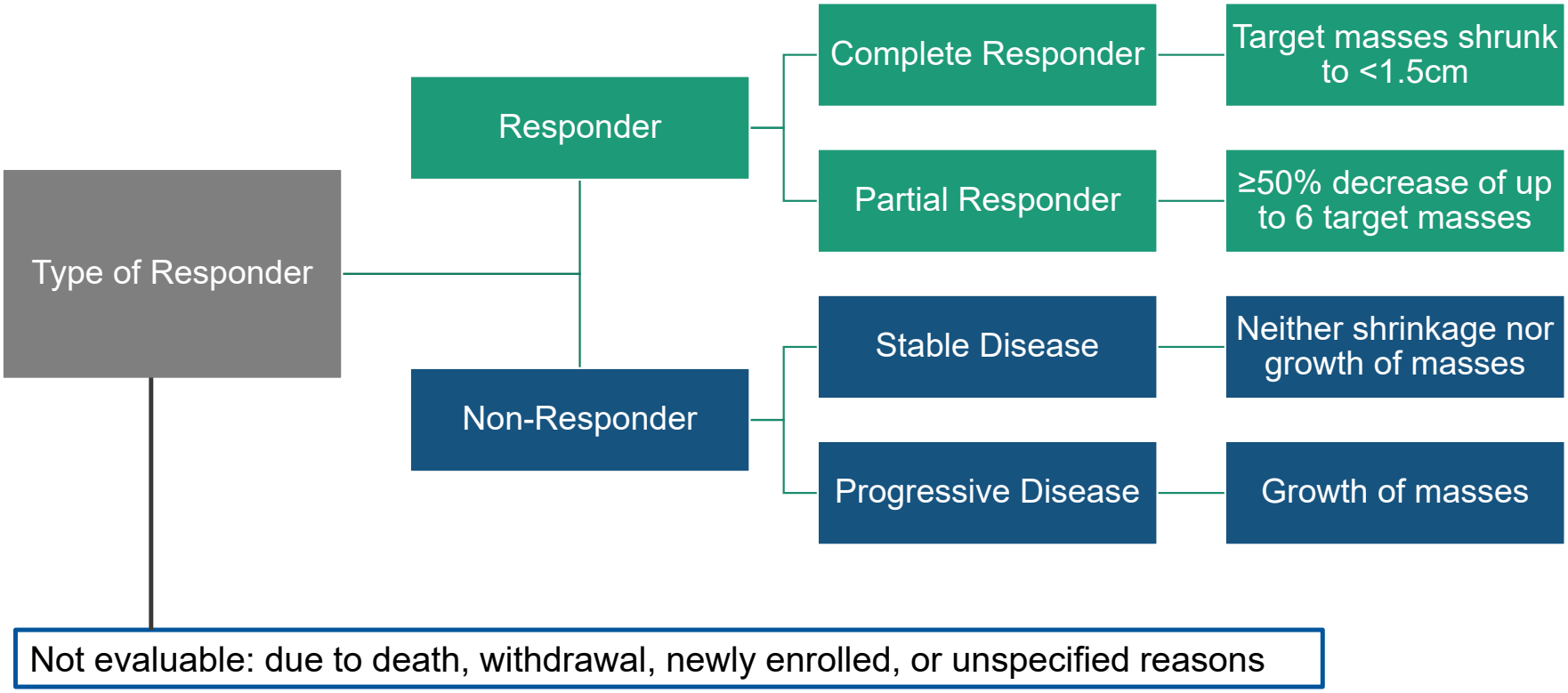
- Phase III multicenter single-arm
- Tabelecleucel administered IV for three doses per 35-day cycle for a minimum of two cycles
- Median number of cycles in trials: HSCT - 3 cycles; SOT - 2 cycles

Baseline Characteristics

- 43 participants
  - 33% HSCT and 67% SOT
- Median age: 49 years
- 44% Female
- 84% White
- 67% diffuse large B-cell lymphoma disease morphology

HSCT: hematopoietic stem cell transplant, IV: intravenous, SOT: solid organ transplant

Response Classifications



cm: centimeters

Response Results

- About half of the participants in the ALLELE trial (22 of 43 participants) had an Objective Response (HSCT: 50%, SOT: 52%).
  - 28% had complete response (43% HSCT, 21% SOT)
- Median time to response: 1 month (IQR: 1 - 2.1)
- Response duration
  - Median duration of response: 23 months (95% CI: 6.8 - NE)
  - 12/22 participants had a response duration greater than six months
- Seven participants not evaluable: three died, one withdrew, two newly enrolled, and one unspecified reasons.

CI: confidence interval, HSCT: hematopoietic stem cell transplant, IQR: interquartile range, NE: not evaluable, SOT: solid organ transplant

Median Overall Survival Results, months

ALLELE

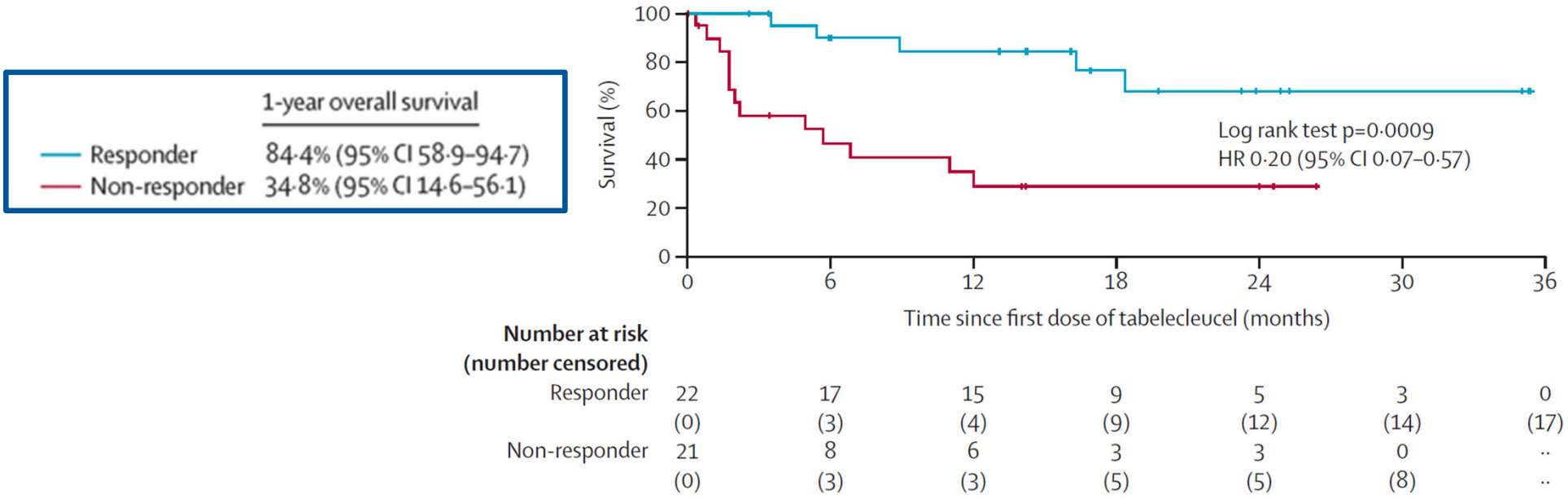
- **Overall**: 18.4 (95% CI: 1.0-26.2)
- **HSCT**: Not reached (95% CI: 5.7-NE)
- **SOT**: 16.4 (95% CI: 5-NE)

Natural History

- **Overall:** 3.3 (95% CI: 2.0, 8.0)
- **HSCT**: 0.7 (95% CI: 0.3-1.0)
- **SOT**: 4.1 (95% CI: 1.9-8.5)

CI: confidence interval, HSCT: hematopoietic stem cell transplant, NE: not estimable, SOT: solid organ transplant

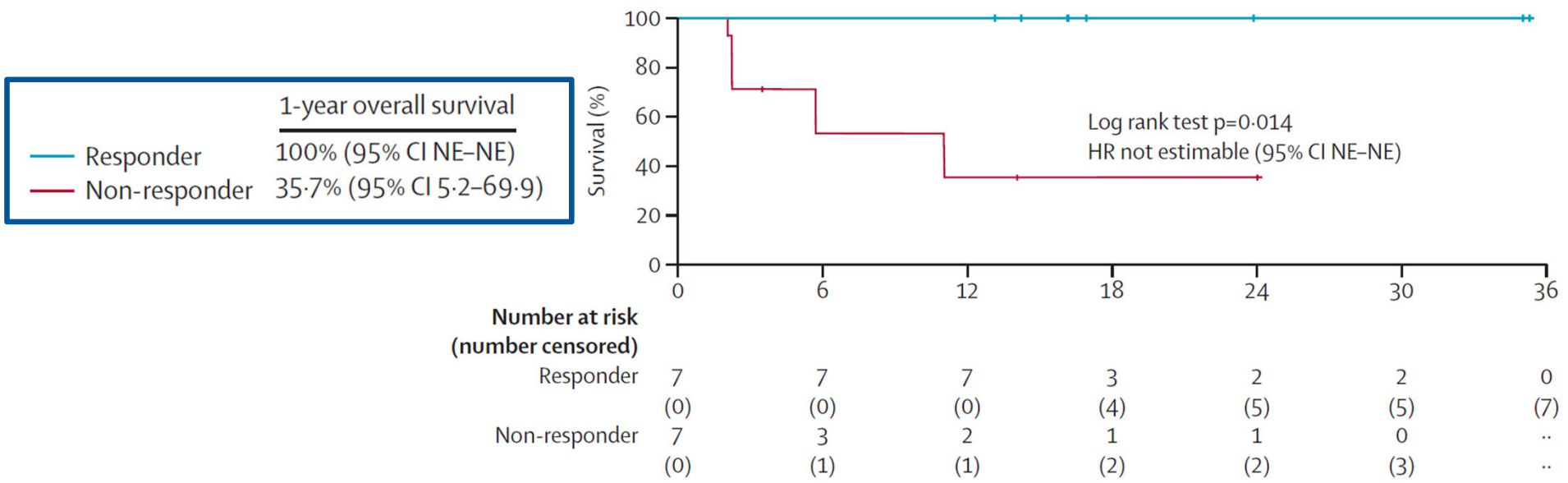
Survival by Response Status: Overall (ALLELE)



CI: Confidence interval, HR: Hazard ratio

Reprinted from The Lancet, Vol. 25. Tabelecleucel for allogeneic haematopoietic stem-cell or solid organ transplant recipients with Epstein–Barr virus-positive post-transplant lymphoproliferative disease after failure of rituximab or rituximab and chemotherapy (ALLELE): a phase 3, multicentre, open-label trial, Page 382., Copyright (2024), with permission from Elsevier.

Survival by Response Status: HSCT (ALLELE)

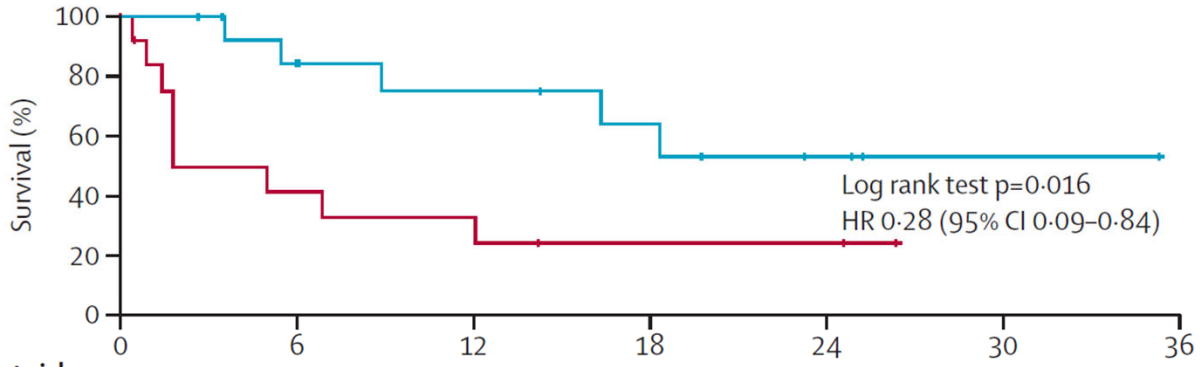


CI: confidence interval, HR: hazard ratio, HSCT: hematopoietic stem cell transplant, NE: not estimable

Reprinted from The Lancet, Vol. 25. Tabelecleucel for allogeneic haematopoietic stem-cell or solid organ transplant recipients with Epstein–Barr virus-positive post-transplant lymphoproliferative disease after failure of rituximab or rituximab and chemotherapy (ALLELE): a phase 3, multicentre, open-label trial, Page 382., Copyright (2024), with permission from Elsevier.

Survival by Response Status: SOT (ALLELE)

	1-year overall survival
Responder	75·2% (95% CI 40·7–91·4)
Non-responder	33·6% (95% CI 10·4–59·1)



Number at risk (number censored)							
Responder	15	10	8	6	3	1	0
	(0)	(3)	(4)	(5)	(7)	(9)	(10)
Non-responder	14	5	4	2	2	0	..
	(0)	(2)	(2)	(3)	(3)	(5)	..

CI: confidence interval, HR: hazard ratio, SOT: solid organ transplant

Reprinted from The Lancet, Vol. 25. Tabelecleucel for allogeneic haematopoietic stem-cell or solid organ transplant recipients with Epstein–Barr virus-positive post-transplant lymphoproliferative disease after failure of rituximab or rituximab and chemotherapy (ALLELE): a phase 3, multicentre, open-label trial, Page 382., Copyright (2024), with permission from Elsevier.

Adverse Events of Special Interest

- Most common AEs: Disease progression, pyrexia, diarrhea, fatigue, and nausea
- Graft-versus-host-disease (GvHD)
  - In ALLELE, one case of acute GvHD occurred in a HSCT participant; judged to be non-serious and unrelated to tabelecleucel.
  - In U.S. EAP & Phase II trials, five other events of acute GvHD were reported
- Tumor flare, cytokine release syndrome, and organ rejection were not observed in the ALLELE trial or other tabelecleucel studies

AE: Adverse event, EAP: expanded access program, HSCT: hematopoietic stem cell transplant, U.S.: United States

Controversies and Uncertainties

- Lack of comparative trial evidence
- Long-term durability is not yet established
- Potentially clinically important differences in subgroup treatment effects
- Additional data needed to confirm safety profile

Benefits Beyond Health and Special Ethical Priorities

Key Points

- Currently, there are limited treatment options and poor survival rates for people living with EBV+ PTLD.
- “Off-the-shelf” T-cell therapy could allow for quicker access to EBV+ PTLD treatment outside specialized academic medical centers.
- Therapy can be delivered in any setting, improving access.

Public Comments Received

- High unmet need associated with R/R EBV+ PTLD due to high cost burden, current disease management options, and poor survival.

Summary

R/R EBV+ PTLD has a poor prognosis without treatment

Treatment with Tabelecleucel led to:

Partial or complete remission observed in a substantial proportion of patients	Extended survival for patients who otherwise have a poor survival rates	Relatively few severe harms

ICER Evidence Ratings for tabelecleucel

Treatment	Comparator	Population	Evidence Rating
Tabelecleucel	Usual Care	SOT or HSCT recipients with R/R EBV+ PTLD after rituximab ± chemotherapy	A

A: high certainty of substantial net health benefit

HSCT: Hematopoietic stem cell transplantation, R/R EBV+ PTLD: relapsed/refractory Epstein-Barr virus positive post transplant lymphoproliferative disease, SOT: Solid organ transplant

Questions?

Tabelecleucel for Epstein-Barr Virus Positive Post-Transplant Lymphoproliferative Disease: Effectiveness and Value

Woojung Lee, PharmD, PhD

Associate Director, Health Economics and Decision Modeling

Institute for Clinical and Economic Review

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Key Review Team Members

Team Role	Assigned Team Member
Modelers	Woojung Lee PharmD, PhD Marina Richardson PhD, MSc

Disclosures

Woojung Lee and Marina Richardson are employees of the Institute for Clinical and Economic Review (ICER).

Woojung Lee and Marina Richardson have no conflicts to disclose defined as more than $10,000 in healthcare company stock or more than $5,000 in honoraria or consultancies relevant to this report during the previous year from health care manufacturers or insurers.

Objective

EBV+ PTLD

To evaluate the lifetime cost-effectiveness of tabelecleucel compared to usual care in people with relapsed or refractory EBV+ PTLD.

Unmet Need

Condition	Absolute evLY Shortfall	Proportional evLY Shortfall
Relapsed/Refractory EBV+ PTLD	26.4	89.5%
Other Example Conditions		
Osteoporosis	2.6	19%
Multiple Myeloma	18.7	95.7%

evLY: equal value of life years gained

Methods in Brief

Methods Overview

Domain	Approach
Model	Markov Model
Setting	United States
Perspective	Health Care Sector Perspective
Time Horizon	Lifetime
Discount Rate	3% per year (costs and outcomes)
Cycle Length	1 month
Primary Outcome	Life years (LYs), Cost per quality-adjusted life years (QALYs) gained; equal value life years (evLYs) gained, Costs

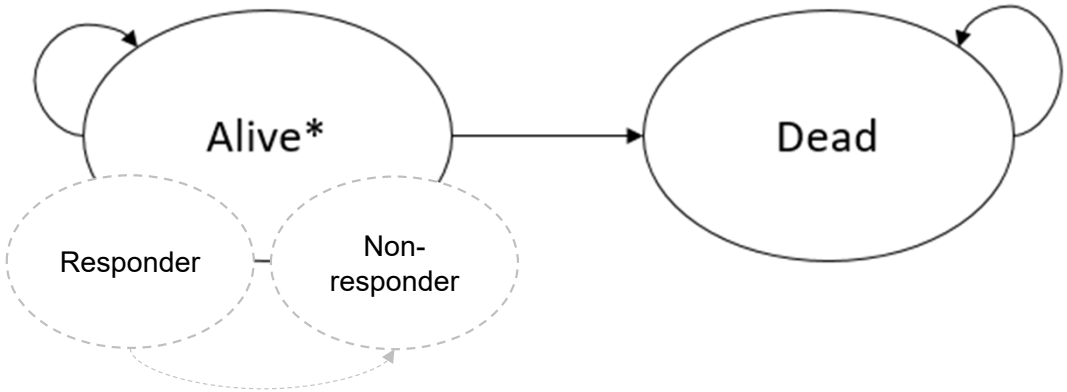
Model Cohort Characteristics

Characteristic	SOT Population (67%)	HSCT Population (33%)	Overall Population†
Mean Age, Years	44.4	51.9	48.5
Female, %	45%	43%	44%
	Mahadeo et al., 2024*		

* Lancet Oncol; 2024; 25(3), 376-387.
† The SOT and HSCT populations account for 67% and 33% of the overall population, respectively.

HSCT: hematopoietic stem-cell transplant, SOT: solid organ transplant

Model Schematic



* Within the alive health state, response status will be tracked.

Patients will be assigned as responders or non-responders at the start of cycle two of the model. After cycle two, a proportion of patients will move from responder to non-responder.

Key Assumptions

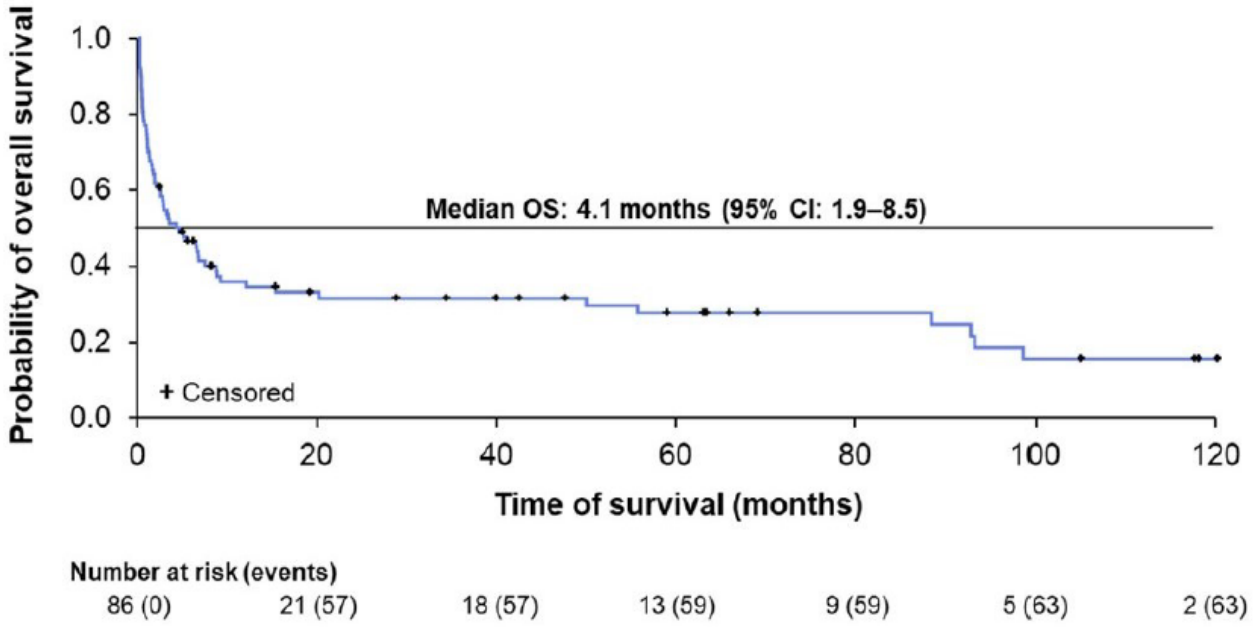
- **Response is defined as complete or partial response**
  - Non-response is defined as stable or progressive disease
- **Mortality and quality of life after 5 years reflect a post-transplant population**
  - Patients who survive for 5 years incur similar health care costs as the general US population
- **Survival benefit of tabelecleucel is the same for the SOT and HSCT populations**
  - Overall survival benefit with tabelecleucel compared to usual care remain constant until year 5
- **The utility values are approximated based a study of large B-cell lymphoma**
  - Utility estimates for a responder and non-responder are based on the estimates for disease-free survival and progressive disease, respectively
- **Chemotherapy costs are represented by CHOP**
  - Varied widely in sensitivity analyses

CHOP: Cyclophosphamide, hydroxydaunorubicin, oncovin (vincristine), and prednisone

Key Model Inputs

Survival, Usual Care

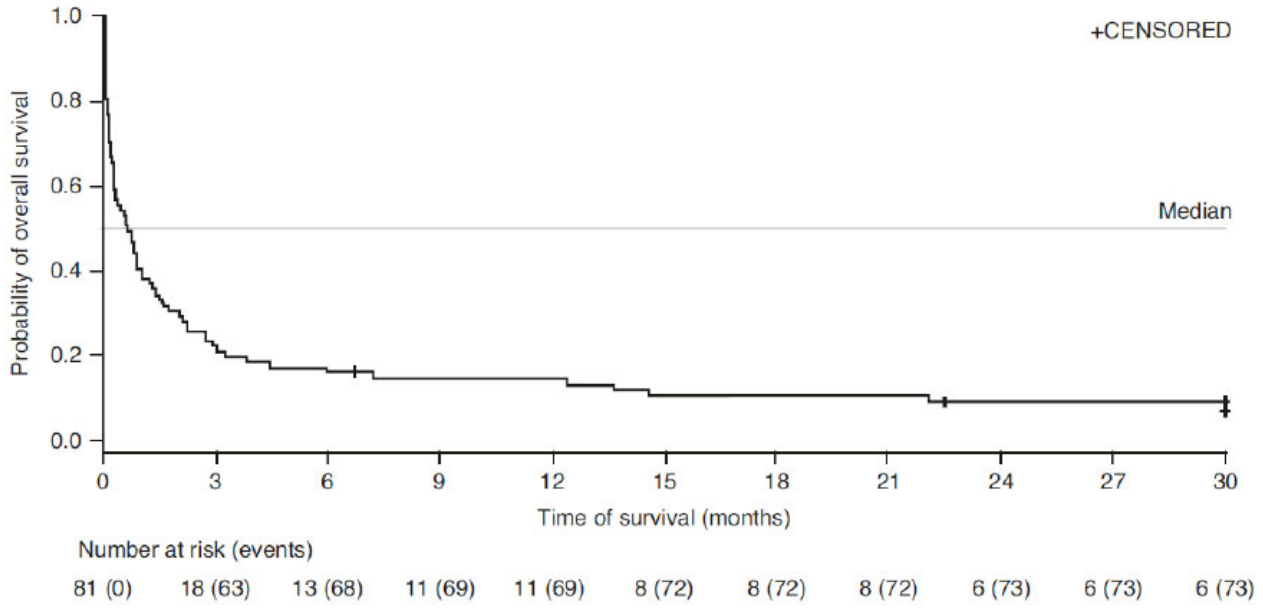
Kaplan Meier Overall Survival Curve for SOT Recipients with Relapsed/Refractory EBV+ PTLD



Source: Dharnidharka et al., 2021, Hemasphere; 2022; 6(Suppl), 997-998

Survival, Usual Care (cont'd)

Kaplan Meier Overall Survival Curve for HSCT Recipients with Relapsed/Refractory EBV+ PTLD



Source: Socié et al., 2024, Bone Marrow Transplant; 2024; 59(1), 52-58

Survival, Tabelecleucel

Survival Estimate	SOT Population	HSCT Population
Hazard Ratio of Overall Survival	0.37 (0.20, 0.71)	0.37 (0.20, 0.71)
Source	Barlev et al., 2024*	

Note: Barlev et al., 2024 appears to include only a subset of the ALLELE study population.
* Journal of Medical Economics; 2024; 1-10.

Response at One Month

SOT Population	Tabelecleucel	Usual Care
Responders	52%	13.5%
HSCT Population	**Tabelecleucel**	**Usual Care**
Responders	50%	13%
Source	Mahadeo et al., 2024*	Estimated based on Socie et al., 2024†, Mahadeo et al., 2024*

Note: The response with usual care in the HSCT population at month 1 was derived using the response at month 6 from Socie 2024 and the ratio between the responses at month 1 and month 6 from Mahadeo 2024. The response with usual care in the SOT population at month 1 was derived using the response ratio between tabelecleucel and usual care in the HSCT population.

* Lancet Oncol; 2024; 25(3), 376-387.

† Bone Marrow Transplant; 2024; 59(1), 52-58.

Response Over Time

	SOT Population	HSCT Population
HR of Overall Survival Between Responders vs. Non-Responders	0.2 (0.07, 0.57)	0.2 (0.07, 0.57)
Source	Mahadeo et al., 2024*	

* Lancet Oncol; 2024; 25(3), 376-387.

	SOT Population	HSCT Population
Monthly Transition Probability of Becoming a Non-Responder	0.17	0.03
Source	Mahadeo et al., 2024*	

* Lancet Oncol; 2024; 25(3), 376-387.

HR: hazard ratio, HSCT: hematopoietic stem-cell transplant, SOT: solid organ transplant

Health State Utilities

Parameter	SOT Population	HSCT Population
Responder	0.83	0.83
Non-Responder	0.39	0.39
Disutility of Adverse Events of Usual Care	-0.15	-0.15
Source	Best et al., 2005*	

* Value in health; 2005; 8(4), 462-470.

HSCT: hematopoietic stem-cell transplant, SOT: solid organ transplant

Drug Utilization, Tabelecleucel

Tabelecleucel	SOT Population	HSCT Population	Source
Number of Cycles	2 (6 doses)	3 (9 doses)	Mahadeo et al., 2024*
Route of Administration	IV	IV	

* Lancet Oncol; 2024; 25(3), 376-387.

HSCT: hematopoietic stem-cell transplant, IV: intravenous, SOT: solid organ transplant

Drug Utilization, Usual Care

Usual Care	SOT Population	HSCT Population
Rituximab Monotherapy	0%	84%
Rituximab + Chemotherapy	100%	16%
Source	Dharnidharka et al., 2021*	Socié et al., 2024†

* Hemasphere; 2022; 6(Suppl), 997-998.
† Bone Marrow Transplant; 2024; 59(1), 52-58.

HSCT: hematopoietic stem-cell transplant, SOT: solid organ transplant

Drug Costs

Intervention‡	Net Price*	Source
Tabelecleucel	$287,500 per 35-day treatment cycle ($95,833 per admin)†	IPD Analytics
Rituximab	$39.75 per 10mg	ASP Pricing File, 2024
Cyclophosphamide	$1.04 per 5mg	
Doxorubicin Hydrochloride	$3.08 per 10mg	
Vincristine Sulfate	$8.01 per mg	
Prednisone	$0.33 per 50mg	REDBOOK

* Mark-up is not included
† Placeholder price
‡ Administration cost of $134 was applied for intravenously administered drugs

Results

Base-Case Results

Drug	Cost*	QALYs	evLYs	Life Years
Tabelecleucel	$986,000	5.7	6.3	8.0
Usual Care	$315,000	2.1	2.1	3.1
Incremental Change	$671,000	3.6	4.2	4.9

*Based on placeholder price

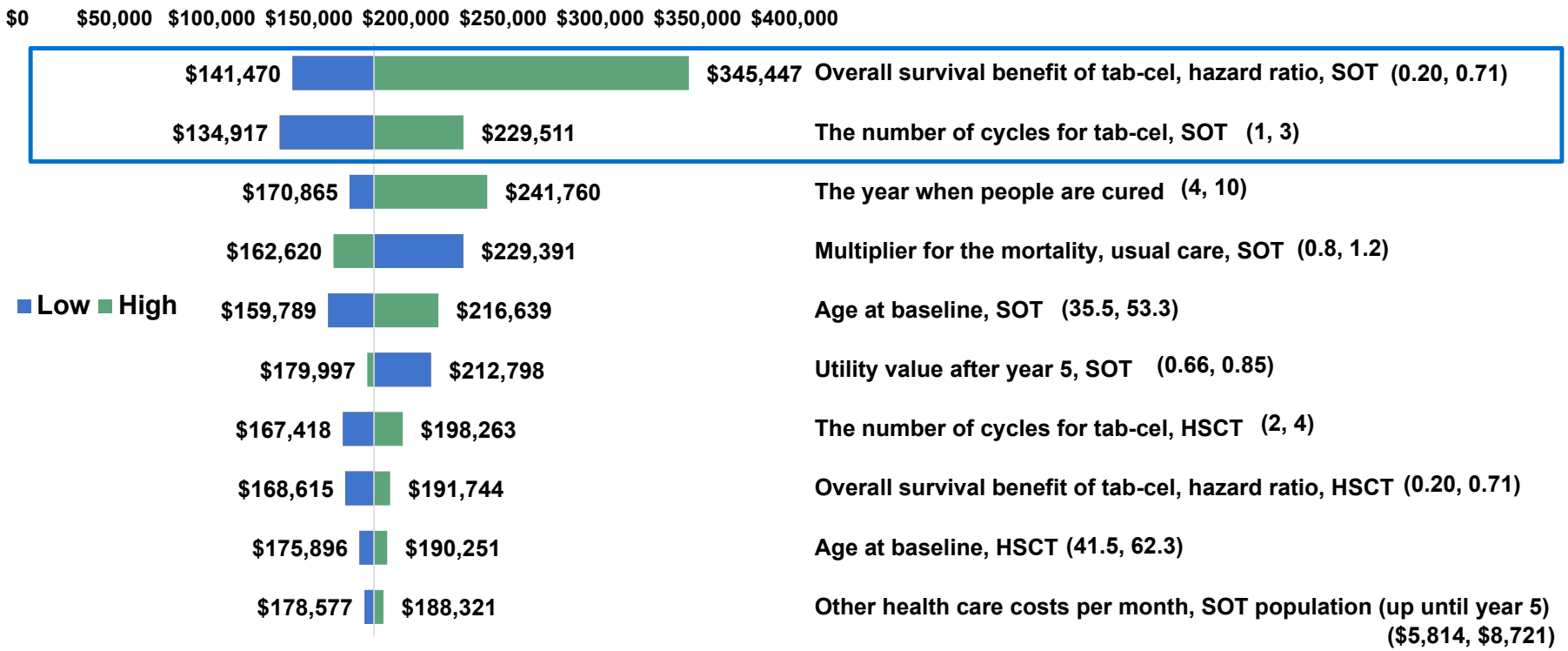
evLY: equal value of life years gained, QALY: quality-adjusted life years

Base-Case Incremental Ratio Results

Treatment	Comparator	Cost per QALY Gained*	Cost per evLY Gained*	Cost per LY Gained
Tabelecleucel	**Usual Care**	$184,000	$157,000	$135,000

* Based on placeholder price

One Way Sensitivity Analyses



Note: The results are based on a placeholder price for tabelecleucel

HSCT: hematopoietic stem-cell transplant, SOT: solid organ transplant

Probabilistic Sensitivity Analysis

Probability of tabelecleucel being cost-effective at different willingness-to-pay thresholds

	$50,000	$100,000	$150,000	$200,000
Per QALY	0.0%	2.5%	29.2%	64.4%
Per evLY	0.0%	6.7%	50.7%	81.6%

Note: The results are based on a placeholder price for tabelecleucel

Key Scenario Analysis

Scenarios	Description
Modified Societal Perspective*	Included domains: • Patient productivity† • Patient time seeking care • Patient consumption costs • Caregiver time required
No Flattening of the Survival Curves for Usual Care	No flattening of the survival curves for usual care in the long-term

* Based on an indirect approach
† Formal labor market, household production, non-household production, and volunteering

Key Scenario Analysis (cont'd)

Scenarios	Cost per QALY Gained†	Cost per evLY Gained†
Base-Case	$184,000	$157,000
Modified Societal Perspective*	$89,000	$76,000
No Flattening of the Survival Curves for Usual Care	$203,000	$173,000

* Based on an indirect approach; includes patient productivity, patient time seeking care, patient consumption costs, and caregiver time required

† Based on a placeholder price

Health Benefit Price Benchmark (HBPB)

	Price Per Cycle* at $100,000 Threshold	Price Per Cycle* at $150,000 Threshold
Per QALY Gained	$143,900	$229,400
Per evLY Gained	$173,400	$273,700

* 35-day treatment cycle that consists of 3 administrations

WAC: wholesale acquisition cost, evLY: equal-value life year, QALY: quality-adjusted life year

Limitations

- Lack of available data to model the heterogeneity in disease progression and treatment responses depending on the morphologies of PTLD or the types of transplant a patient has received
- Lack of data on the adjusted clinical benefits of tabelecleucel due to the absence of a randomized clinical trial
- High variability and uncertainty in the composition of usual care

Comments Received

- The costs and quality of life impacts associated with organ/graft rejection among patients with EBV+ PTLD who are treated with chemotherapy
  - Clinical experts have suggested that these events do not have a causal relationship with chemotherapy use.
  - The available data does not provide sufficient evidence to suggest that the incidence of organ/graft rejection differs between the two arms.

Conclusions

- At a placeholder price of $287,500 per 35-day treatment cycle, the incremental cost-effectiveness ratio of tabelecleucel exceeded the commonly accepted willingness-to-pay thresholds.
- The cost-effectiveness of tabelecleucel is largely dependent on the expected survival benefit and the actual drug price.

Questions?

Manufacturer Public Comment and Discussion

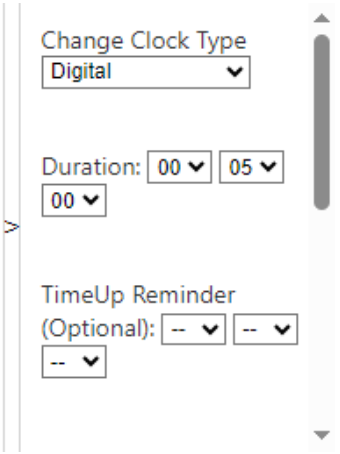
Lara Cavalli, PharmD
VP, Head of Medical Affairs, Pierre Fabre Pharmaceuticals

Conflicts of Interest:

- *Dr. Cavalli is a full-time employee of Pierre Fabre Pharmaceuticals*
- *Dr. Cavalli has collaborated with*

Syneos Health to compose this public comment.

00 : 05 : 00



Lunch

Meeting will resume at 12:45 PM ET

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Voting Questions

Patient Population for all questions:
People with relapsed/refractory Epstein-Barr virus-positive post-transplant lymphoproliferative disease (EBV+ PTLD), who have received at least one prior therapy.

Clinical Evidence

slido

1. Is the current evidence adequate to demonstrate that the net health benefit of tabelecleucel is superior to that provided by usual care?

ⓘ Start presenting to display the poll results on this slide.

Benefits Beyond Health and Special Ethical Priorities

To help inform judgments of overall long-term value for money, please indicate your level of agreement with the following statements:

slido

2. There is substantial unmet need despite currently available treatments.

ⓘ Start presenting to display the poll results on this slide.

slido

3. This condition is of substantial relevance for people from a racial/ethnic group who have not been equitably served by the healthcare system.

ⓘ Start presenting to display the poll results on this slide.

To help inform judgments of overall long-term value for money, please indicate your level of agreement with the following statements based on the relative effects of tabelecleucel versus usual care:

slido

4. The treatment is likely to produce substantial improvement in caregivers' quality of life and/or ability to pursue their own education, work, and family life.

ⓘ Start presenting to display the poll results on this slide.

slido

5. The treatment offers a substantial opportunity to improve access to effective treatment by means of its mechanism of action or method of delivery.

ⓘ Start presenting to display the poll results on this slide.

Break

Meeting will resume at 2:00 PM ET

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Policy Roundtable

Policy Roundtable

Participant	Conflict of Interest
Upton Allen, O.Ont., CD, MBBS, MSc, FAAP, FRCPC, Hon FRCP (UK), FIDSA, Chief, Division of Infectious Disease, The Hospital for Sick Children; Senior Associate Scientist, Research Institute, The Hospital for Sick Children; Professor, Pediatrics, University of Toronto	No conflicts to disclose.
Joseph A. Kopec, Patient	No conflicts to disclose.
Sarah Nikiforow MD, PhD, Technical Director, Immune Effector Cell Program, Dana-Farber Cancer Institute	Dr. Sarah Nikiforow served as a PI at Dana-Farber Cancer Center for Tabelecleucel on Atara Biotherapeutics studies (CTL 201, CTL 901, CTL 302, CTL 301, CTL 205), but she did not accept any salary support or payment for serving as PI.
Melissa Pozotrigo, PharmD, BCOP, Senior Clinical Oncology Pharmacist, Oncohealth	Dr. Pozotrigo is a full-time employee at Oncohealth.
Emily Tsiao, PharmD, BCPS, Medical Policies Clinical Pharmacist, Premera Blue Cross	Dr. Tsiao is a full-time employee at Premera Blue Cross.
Douglas Worthen, Patient	No conflicts to disclose.

NE CEPAC Council Reflections

Next Steps

- Meeting recording posted to ICER website next week
- Final Report published on or around December 16, 2024
    - Includes description of NE CEPAC votes, deliberation, policy roundtable discussion
- Materials available at: https://icer.org/assessment/ebv-ptld-2024/

Adjourn

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