Overall and progression-free survival in patients with Epstein-Barr virus–driven post-transplant lymphoproliferative disease (EBV+ PTLD) following tabelecleucel treatment: Results from the phase 3 ALLELE trial

Poster #: CT - 278

Sylvain Choquet[1], Armin Ghobadi[2], Sunita Nasta[3], Sridhar Chaganti[4], Sarah Nikiforow[5], Amer Beitinjaneh[6], Sonali Chaudhury[7], Rabi Hanna[8], Robert Baiocchi[9], Glen Lew[10], Ashley Swanson[10], Sandrine Roye[11], Daan Dierickx[12], and Susan Prockop[13]

1. University Hospital Pitié Salpêtrière, Paris, France. 2. Washington University, St Louis, MO, United States. 3. University of Pennsylvania, Philadelphia, PA, United States. 4. Centre for Clinical Haematology, University Hospitals Birmingham, Birmingham, United Kingdom. 5. Dana-Farber Cancer Institute, Boston, MA, United States. 6. University of Miami/Jackson Memorial Hospital, Miami, FL, United States. 7. Lurie Children's Hospital, Chicago, IL, United States. 8. Cleveland Clinic, Cleveland, OH, United States. 9. James Comprehensive Cancer Center, The Ohio State University, Columbus, OH, United States. 10. Pierre Fabre Pharmaceuticals, Secaucus, NJ, United States. 11. Institut de Recherche Pierre Fabre, Toulouse, France. 12. University Hospitals Leuven, Leuven, Belgium. 13. Dana-Farber/Boston Children's Hospital Cancer and Blood Disorders Center, Boston, MA, United States.

INTRODUCTION

- EBV+ PTLD is a rare complication following SOT or HCT.[1,2]
- Median OS in patients with relapsed/refractory EBV+ PTLD is 0.7 months after HCT and 4.1 months after SOT, representing an unmet therapeutic need.[1,2]
- Tabelecleucel is an off-the-shelf, allogeneic EBV-specific cytotoxic T cell immunotherapy approved in 32 countries, including countries in the EU and UK, as monotherapy for relapsed/refractory EBV+ PTLD from 2 years of age and older.[3,4] Approval was based on the ongoing ALLELE trial (NCT03394365).[5]

Tabelecleucel is manufactured from PBMCs from healthy EBV+ donors. Repeated exposure to EBV induces EBV-transformed BLCLs presenting a broad range of EBV antigens. These BLCLs are co-cultured with donor T cells inducing a polyclonal T-cell population. HLA restriction to EBV is confirmed in the resulting **EBV-specific cytotoxic T lymphocytes**. Cells meeting thresholds for EBV-specific cytotoxicity, alloreactivity, and release criteria are harvested, aliquoted into **single-use vials, cryopreserved, and stored for off-the-shelf use**.[6]

The primary endpoint in terms of ORR was met at an earlier analysis[5]; however, the study is ongoing and the data continue to mature.

Here we report updated results from ALLELE with 89 patients and median of follow-up of 12.7 months, focusing on OS and PFS (data cutoff Sept 1, 2025).

METHODS

- **ALLELE is a multicenter, open-label, phase 3 trial**. Tabelecleucel (2×10^6 cells/kg) is administered IV on days 1, 8, and 15 with clinical and radiographic assessments before the end of each 35-day cycle. Treatment response is evaluated by IORA.
- **Survival status** is being assessed for up to 5 years.
- **Primary efficacy endpoint:** ORR in participants who had received up to 2 tabelecleucel lots with different HLA restrictions.
- **Secondary endpoints:** ORR in the SOT and HCT groups, DOR, OS, the rate of allograft loss or rejection in the SOT group, and safety.
- **Exploratory endpoint:** PFS.
- **Kaplan-Meier methods** for time-to-event endpoints.

Study Participants

- 90 participants were enrolled and 89 treated; 57 with SOT and 32 with HCT.
- 72.2% (n=65) were enrolled at US sites.
- Follow-up was continuing in 21 participants (23.3%) at data cutoff.

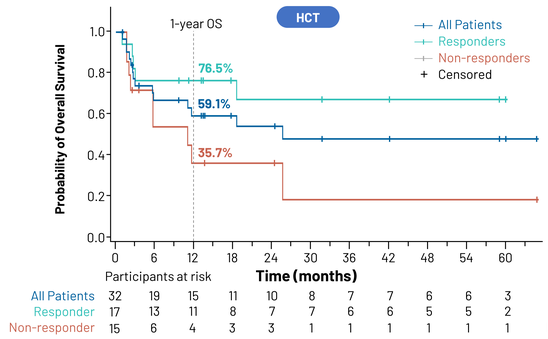
Demographics and disease characteristics	HCT (n=32)	SOT (n=57)	Total (N=89)
Age, median (range), years	49.5 (3.2–73.2)	41.1 (2.7–81.5)	42.8 (2.7–81.5)
Male sex, n (%)	19 (59.4)	33 (57.9)	52 (58.4)
ECOG PS ≥2 (age ≥16 years[a]), n (%)	7 (23.3)	14 (28.6)	21 (26.6)
Lansky score (<16 years[a]), median (range)	85.0 (80.0–90.0)	70.0 (40.0–90.0)	70.0 (40.0–90.0)
PTLD-adapted prognostic index[b] (age ≥16 years[a]), n (%)			
Low risk	1 (3.3)	5 (10.2)	6 (7.6)
Intermediate risk	15 (50.0)	24 (49.0)	39 (49.4)
High risk	14 (46.7)	19 (38.8)	33 (41.8)
Unknown	0 (0.0)	1 (2.0)	1 (1.3)
PTLD morphology, n (%)			
Diffuse large B-cell lymphoma	20 (62.5)	40 (70.2)	60 (67.4)
Plasmablastic lymphoma	1 (3.1)	2 (3.5)	3 (3.4)
Other	11 (34.4)	15 (26.3)	26 (29.2)
Extranodal disease, n (%)	20 (62.5)	46 (80.7)	66 (74.2)
Median (range) time, mo			
From EBV+ PTLD diagnosis to first tabelecleucel infusion	2.27 (0.6–28.1)	7.12 (1.2–190.5)	4.44 (0.6–190.5)
Treatment and prior therapies			
Number of prior systemic treatment lines, median (range)	1.0 (1.0– 4.0)	1.0 (1.0– 5.0)	1.0 (1.0– 5.0)
Prior rituximab only, n (%)	32 (100)	46 (80.7)	78 (87.6)
Prior rituximab + CT, n (%)	1 (3.1)	33 (57.9)	34 (38.2)
Prior immunotherapy, n (%)	1 (3.1)	2 (3.5)	3 (3.4)

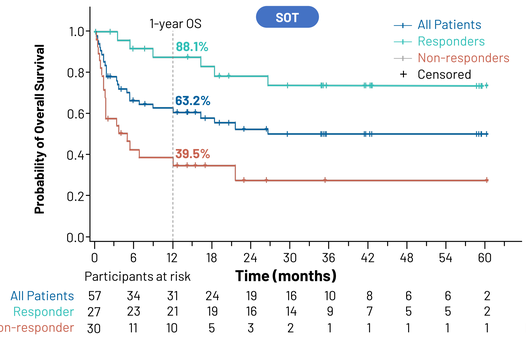
[a]≥16 yrs: n=30 in HCT group, n=49 in SOT group, n=79 overall. <16 yrs: n=2, 8 and 10, respectively. [b]PTLD-adapted prognostic index: low risk (no high-risk factors among age, ECOG PS, and lactate dehydrogenase), intermediate risk (1 HR factor), high risk (2 or 3 HR factors).[7]

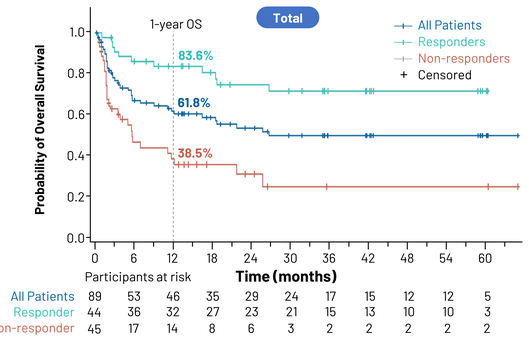
EFFICACY

- ORR (95% CI) in the FAS was 49.4% (38.7–60.2): 53.1% (34.7–70.9) for HCT and 47.4% (34.0–61.0) for SOT. Median OS (95% CI) was 26.6 months (12.1–NE), 25.6 months (5.7–NE), and NE (9.0–NE), respectively. In responders, OS was NE in all groups. Median PFS (95% CI) in responders was 25.6 months (16.9–NE), with a 1-year PFS rate of 75.0% (58.2–85.8).

Kaplan-Meier plots of OS by IORA overall, in responders and non-responders



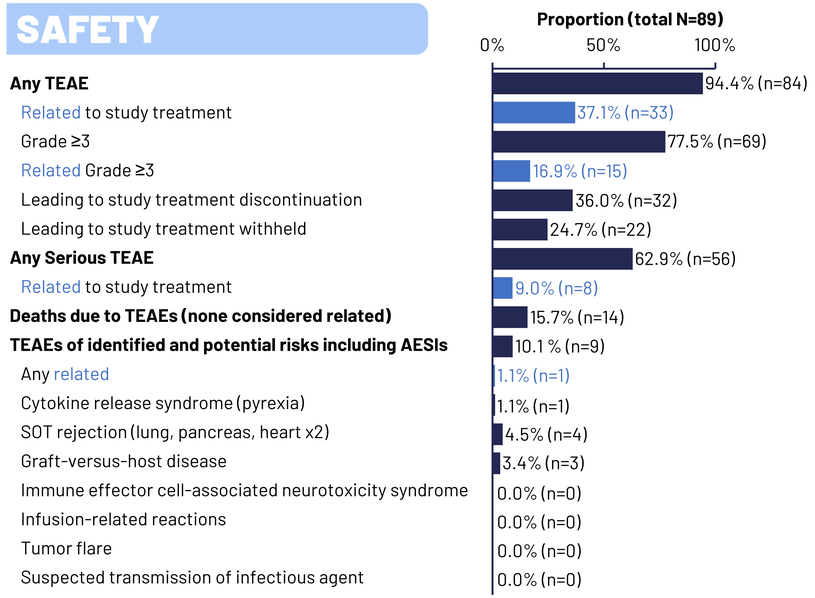




	HCT (n=32)	SOT (n=57)	Total (N=89)
ORR (95% CI)	53.1% (34.7–70.9)	47.4% (34.0–61.0)	49.4% (38.7–60.2)
DOR (95% CI) in responders, mo	23.0 (2.0–NE)	23.3 (15.7–NE)	23.0 (15.9–NE)
Median PFS (95% CI), mo			
All	2.9 (2.0–23.9)	4.4 (1.6–19.5)	3.3 (2.2–19.5)
Responders	23.9 (2.9–NE)	26.6 (16.6–NE)	25.6 (16.9–NE)
PFS at 1 year in responders	76.5%	74.0%	75.0%
Median OS (95% CI), mo			
All	25.6 (5.7–NE)	NE (9.0–NE)	26.6 (12.1–NE)
Responders	NE (2.9–NE)	NE	NE
OS (95% CI) at 1 year	59.1% (39.2–74.5)	63.2% (48.9–74.5)	61.8% (50.4–71.3)
OS (95% CI) at 1 year in responders	76.5% (48.8–90.4)	88.1% (67.5–96.0)	83.6% (68.7–91.8)

Kaplan-Meier plots of PFS by IORA overall, in responders and non-responders

SAFETY



Related Grade ≥3 TEAEs were reported for 16.9% and treatment-related serious TEAEs for 9.0% of patients.

One patient had Grade 1 fever deemed a sign of cytokine release syndrome possibly related to treatment.

No treatment-related deaths were reported.

Cases of graft vs. host disease were reported in 3 HCT patients and transplant rejection in 4 SOT patients; none treatment-related.

There were no reports of infusion-related reactions, immune effector cell-associated neurotoxicity syndrome, tumor flare, HCT rejection, and no suspected transmission of infectious agent.

CONCLUSIONS

- Results from ALLELE show substantial OS and PFS benefit with tabelecleucel in patients with relapsed or refractory EBV+ PTLD, who otherwise have a poor probability of survival.
- ORR and safety data are consistent with previous reports, supporting tabelecleucel as a novel treatment option for relapsed or refractory EBV+ PTLD.

REFERENCES

1. Dharnidharka V, et al. *Blood*. 2021; 138: 2528. **2.** Socié G, et al. *Bone Marrow Transplant*. 2024; 59(1): 52-8. **3.** Keam SJ. *Mol Diagn Ther*. 2023; 27(3): 425-31. **4.** https://www.onclive.com/view/ fda-grants-priority-review-to-resubmitted-bla-for-tabelecleucel-in-ebv-post-transplant-lymphoproliferative-disease. **5.** Mahadeo KM, et al. *Lancet Oncol*. 2024; 25(3): 376-87. **6.** Nikiforow S, et al. *Blood*. 2024; 8(12):3001-12. **7**. Choquet S, et al. *Ann Hematol*. 2007;86(8):599-607.

ABBREVIATIONS

AESI, adverse event of special interest; **BLCL,** EBV-transformed B lymphoblastoid cell lines; **CI,** confidence interval; **CT,** chemotherapy; **DOR,** duration of response; **EBV,** Epstein-Barr virus; **ECOG PS,** Eastern Cooperative Oncology Group performance status; **FAS,** full analysis set; **HCT,** hematopoietic stem cell transplant; **HLA,** human leukocyte antigen; **HR,** hazard ratio; **IORA,** independent oncologic response adjudication; **IV,** intravenously; **N/n,** total number/number of participants; **NE,** not evaluable; **ORR,** overall response rate; **OS,** overall survival; **PBMC,** peripheral blood mononuclear cells; **PFS,** progression free survival; **PTLD:** post-transplant lymphoproliferative disease; **Q1, Q3**, first and third quartiles; **SOT,** solid organ transplant; **TEAE,** treatment-emergent adverse event.

ACKNOWLEDGEMENTS

We thank all participants and investigators for their valuable and continuing contributions. We thank Jamie Benson, PhD, of Red Nucleus, for editorial assistance, coordination, and writing support, on behalf of Pierre Fabre.

Contact: Sylvain Choquet, sylvain.choquet@aphp.fr

Presented at: 14th SOHO Annual Meeting, September 9–12, 2026, Houston, Texas