(PF1029) OVERALL AND PROGRESSION-FREE SURVIVAL IN PATIENTS WITH EPSTEIN-BARR VIRUS-DRIVEN POST-TRANSPLANT LYMPHOPROLIFERATIVE DISEASE (EBV+ PTLD) TREATED WITH TABELECLEUCEL IN THE PHASE 3 ALLELE TRIAL

Topic: 20. Other aggressive lymphomas

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Background

Median overall survival (OS) in patients with relapsed/refractory Epstein-Barr virus-associated post-transplant lymphoproliferative disease (R/R EBV+ PTLD) is 0.7 months after hematopoietic cell transplant (HCT) and 4.1 months after solid organ transplant (SOT), representing an unmet therapeutic need (Socié et al. Bone Marrow Transplant, 2024; Dharnidharka et al. Blood, 2021). Tabelecleucel is an off-the-shelf, allogeneic EBV-specific cytotoxic T cell therapy being investigated in patients with R/R EBV+ PTLD after HCT or SOT in the ongoing ALLELE trial (NCT03394365). Results from 86 patients have been reported (Nikiforow et al. ASH 2025).

Aims

To present updated results from ALLELE with 89 patients and 12.7 months of follow-up, focusing on OS and progression-free survival (PFS).

Methods

ALLELE is a multicenter, open-label, phase 3 trial. Patients received intravenous tabelecleucel at 2 x 10^6 cells/kg on days 1, 8, and 15 in 35-day cycles. Disease assessments were conducted at the end of each cycle and treatment response evaluated by independent oncologic response adjudication (IORA). Survival status was assessed for up to 5 years. All eligible patients received tabelecleucel, and efficacy and safety were evaluated in all who received ≥1 dose. The primary endpoint was objective response rate (ORR). Other endpoints included OS and PFS.

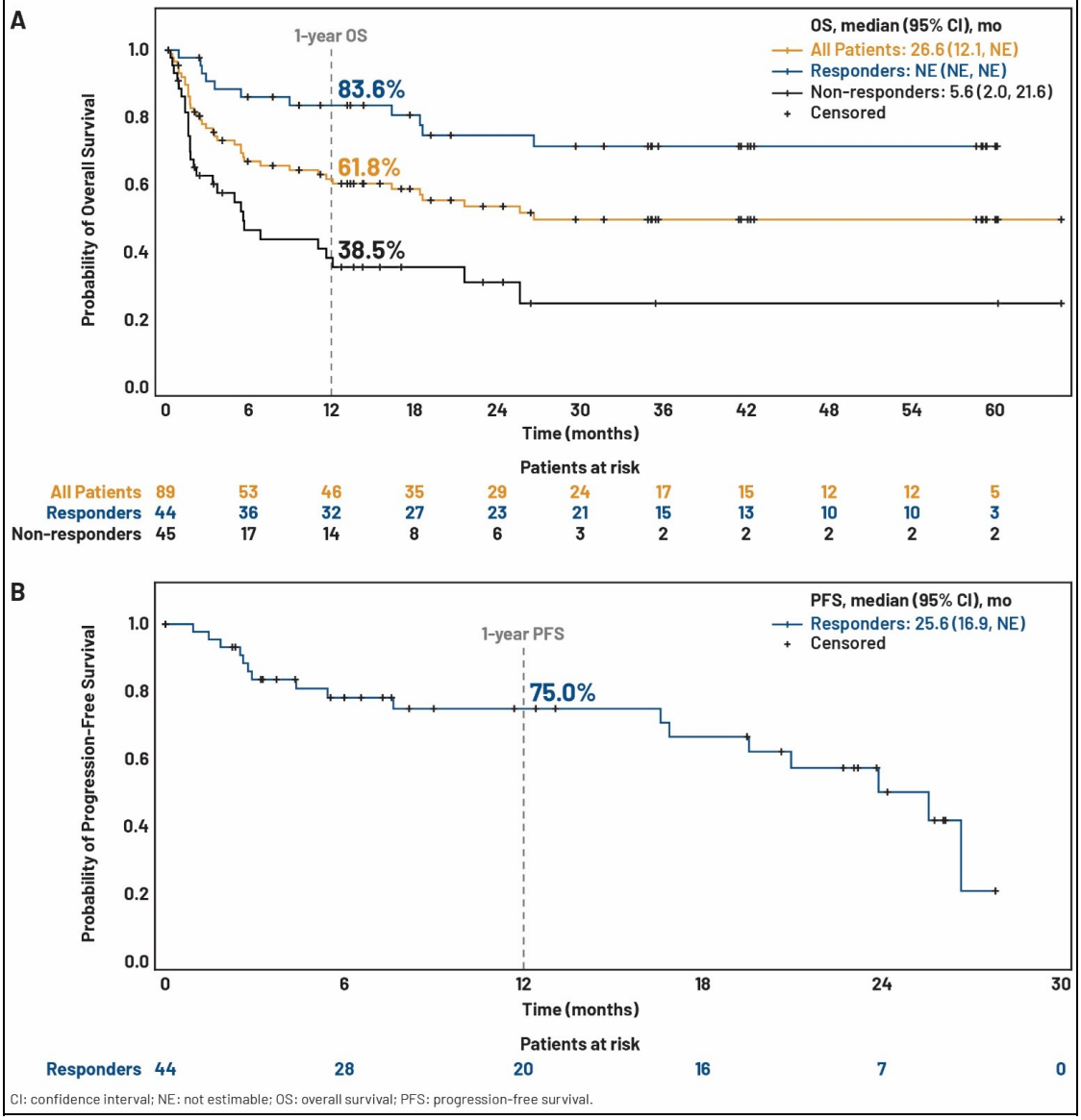
Results

As of 1 September 2025, 90 patients were enrolled and 89 received tabelecleucel (32 HCT, 57 SOT). The median (range) age was 42.8 (2.7–81.5) years and 52/89 (58.4%) were male. The overall ORR was 49.4% (44/89, 95% CI 38.7, 60.2). ORR for HCT was 53.1% (17/32, 95% CI 34.7, 70.9) and for SOT was 47.4% (27/57, 95% CI 34.0, 61.0). After a median follow-up time of 12.7 months, the overall median OS (95% CI) was 26.6 months (12.1, not estimable [NE]; Figure 1A). Median OS (95% CI) in HCT was 25.6 months (5.7, NE) and in SOT was NE (9.0, NE). In patients responding to treatment, median OS (95% CI) was NE (NE, NE) overall (Figure 1A), NE (2.9, NE) for HCT, and NE (NE, NE) for SOT. In all patients, the 1-year OS rate (95% CI) was 61.8% (50.4, 71.3) overall, 59.1% (39.2, 74.5) for HCT, and 63.2% (48.9, 74.5) for SOT. In responders, the 1-year OS rate (95% CI) was 83.6% (68.7, 91.8) overall, 76.5% (48.8, 90.4) for HCT, and 88.1% (67.5, 96.0) for SOT. The median duration of response (95% CI) in responders was 23.0 months (15.9, NE). Median PFS (95% CI) in responders was 25.6 months (16.9, NE), with a 1-year PFS rate (95% CI) of 75.0% (58.2, 85.8; Figure 1B). Treatment-related treatment emergent adverse events (TEAEs) of grade ≥3 were reported in 15 patients (16.9%) and treatment-related serious TEAEs in 8 (9.0%). One patient was reported with 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 (9.4%), and transplant rejection in 4 SOT patients (7.0%), none of which were treatment-related. There were no reports of infusion-related reactions, immune effector cell-associated neurotoxicity syndrome, tumor flare, immunogenicity events, or HCT rejection.

Summary/Conclusion

Results from ALLELE show OS and PFS benefit with tabelecleucel in patients with R/R 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 R/R EBV+ PTLD.

Figure 1. Kaplan-Meier plots of (A) overall survival in the full analysis set, responders, and non-responders, and (B) progression-free survival in responders.



CI: confidence interval; NE: not estimable; OS: overall survival; PFS: progression-free survival.