# (PF1041) TABELECLEUCEL FOR ALLOGENEIC OR SOLID ORGAN TRANSPLANT RECIPIENTS WITH EBV+ PTLD AFTER FAILURE OF RITUXIMAB OR RITUXIMAB PLUS CHEMOTHERAPY: PAEDIATRIC SUBGROUP ANALYSIS OF THE PHASE 3 ALLELE STUDY

**Topic:** 20. Other aggressive lymphomas

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## Background

Epstein–Barr virus-associated post-transplant lymphoproliferative disease (EBV+ PTLD) is an ultra-rare complication with low survival if initial treatment fails. Tabelecleucel is an off-the-shelf allogenic EBV-specific cytotoxic T cell therapy. Results in the overall population have been presented previously (Ghobadi et al. ASH 2024).

## Aims

We report results from 12 paediatric patients who have participated in the ALLELE trial.

## Methods

ALLELE is a multicentre, open-label Phase 3 trial including patients of any age. Patients received intravenous tabelecleucel at 2 x $10^6$ cells/kg on days 1, 8, and 15 in 35-day cycles. Response was evaluated by independent oncologic response adjudication based on clinical and radiographic evidence. After treatment, disease status was evaluated for up to 2 years and survival status up to 5 years. The primary objective was objective response rate (ORR). Secondary objectives included rates of complete response (CR), partial response (PR), time to response (TTR), duration of response (DOR), and overall survival (OS). Safety analyses were conducted in all patients who received ≥1 dose of tabelecleucel.

**Results**

At data cutoff on 10 September 2024, 86 patients ranging in age from 2.7–81.5 years were enrolled and treated with tabelecleucel. Among patients, 12 were <17 years of age, 3 had hematopoietic cell transplantation (HCT) and 9 solid organ transplant (SOT). The median (range) age of paediatric patients was 8.8 (2.7–16.8) years. Two patients were aged 2–<6 years, 5 were 6–<12 years and 5 were 12–<17 years. At the time of initial PTLD diagnosis, there were 9 patients with stage III (n=4) or stage IV (n=5) disease (Lugano classification, LYRIC modification), and 3 with unknown stage. All 12 patients had extranodal disease and 8 had lymph node disease at screening. The median (range) time from PTLD diagnosis to first tabelecleucel dose was 3.0 (0.6–43.8) months. The ORR in paediatric patients was 50.0% (6/12 patients): CR was reported in 4/12 patients (1 HCT and 3 SOT), and PR was observed in 2/12 patients (1 HCT and 1 SOT). For the 6 responders, the median (range) TTR was 1.6 (0.9–4.7) months. Two of the responders had a durable response of >6 months. At the time of data cutoff, 3 of the responders were censored with no disease progression or death. After a median (range) follow-up time of 5.2 (1.1–52.7) months, at the time of data cutoff, 50% (6/12) of patients were still alive, including 5 responders and 1 non-responder. Six patients developed treatment-emergent serious adverse events (TESAEs). Fatal SAEs were reported in 2 patients, none were related to treatment. 4 TESAEs (in 2 patients) were considered treatment-related. One SOT patient reported grade 1 pyrexia, considered a sign of possible treatment-related cytokine release syndrome. There were no reports of tumour flare reactions, infusion reactions, immune effector cell associated neurotoxicity, transmission of infectious agent, graft vs host disease, or transplant rejection. Tabelecleucel showed a favourable safety profile in the paediatric population.

**Summary/Conclusion**

This analysis of 12 paediatric patients from the ALLELE trial suggests that the risk/benefit profile in the paediatric subgroup is consistent with that reported in the overall population. These findings support the use of tabelecleucel in paediatric patients with refractory/relapsed EBV+ PTLD who have poor survival and limited treatment options.