Public Comment: 12-Month Update on Tabelecleucel for EBV+ PTLD
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Thank you for the opportunity to provide this 12-month update following ICER's December 2024 final report on tabelecleucel for relapsed/refractory Epstein–Barr virus-driven post-transplant lymphoproliferative disease (R/R EBV+ PTLD). EBV+ PTLD remains a rare but potentially fatal complication of transplantation, with limited treatment options. We are pleased to share new clinical data that further inform the safety and efficacy profile of tabelecleucel. Previously, the results from the ALLELE October 2023 data cut were reported (N=75). Here we report on the September 2024 data cut, with additional patients (N=86), and including a pediatric subgroup analysis, which was reported for the first time at ASH 2025.

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1. Subgroup Analysis Based on Prior Treatment[1]
Updated results from the Phase 3 ALLELE trial focused on patients with R/R EBV+ PTLD after hematopoietic cell transplant (HCT) or solid organ transplant (SOT), analyzed by prior treatment.

- **Efficacy:** Objective response rates (ORR) remain consistent with previous findings: 48.3% in HCT (n=29) and 47.4% in SOT (n=57). Subgroup analyses show ORRs of 52.4% for SOT patients who failed rituximab alone (n=11/21) and 44.4% for those who failed rituximab plus chemotherapy (n=16/36). Median time to response was 1.0 month for HCT responders and 2.1 months for SOT responders. Survival outcomes remain encouraging, with estimated 1-year overall survival rates of 54.3% (HCT) and 62.6% (SOT). Among treatment responders, 1-year survival rates were 71.4% (HCT) and 88.1% (SOT).
- **Safety:** Serious adverse events (SAEs) were reported in 58.6% of HCT and 66.7% of SOT patients, with treatment-related SAEs in only 3.4% and 12.3% of HCT and SOT patients, respectively. Events of special interest, including GvHD and transplant rejection, were infrequent and were not considered to be treatment-related.

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2. Subgroup Analysis in Pediatric Patients[2]
Outcomes in pediatric patients from the ALLELE trial; data cutoff was September 2024, with 12 patients <17 years of age (3 HCT, 9 SOT) included in this analysis.

- **Efficacy:** Among 12 pediatric patients treated, the ORR was 50% (6/12), including 4 complete (1 HCT, 3 SOT) and 2 partial (1 HCT, 1 SOT) responses, which is consistent with the ORR for the overall study population (47.7%). At the data cutoff, 5 of 6 responders were alive. The efficacy profile in pediatric patients closely mirrors that of the overall population, supporting the use of tabelecleucel in this high-risk subgroup.

- **Safety:** The safety profile in pediatric patients consistent with reports from the overall study population, with no new safety signals identified. 6 patients developed treatment-emergent SEAs, 4 were considered treatment-related. 1 SOT patient reported a grade 1 fever, which was considered a sign of possible treatment-related CRS, with no signs of severity. There were no other reports of AEs of special interest (including no reports of GvHD or transplant rejection).

Summary
New data published in the 2025 confirm that tabelecleucel maintains a consistent efficacy and safety profile in both adult and pediatric patients with R/R EBV+ PTLD. These findings align closely with outcomes previously reported, reinforcing the role of tabelecleucel as a valuable therapeutic option for this ultra-rare and challenging condition.

References

1. Nikiforow S, Mahadeo K, Chaganti S, et al. (2025, December 6-9). *Subgroup analysis based on prior treatment from the Phase 3 ALLELE study of tabelecleucel for Epstein-Barr virus-driven post-transplant lymphoproliferative disease*. [Poster presentation]. American Society of Hematology, Orlando, FL, USA. Abstract 1934.
2. Chaudhury S, Reshef R, Nikiforow S, et al. (2025, December 6-9). *Subgroup analysis in pediatric patients from the Phase 3 study of tabelecleucel for allogeneic or solid organ transplant recipients with Epstein-Barr virus-driven post-transplant lymphoproliferative disease after failure of rituximab or rituximab and chemotherapy (ALLELE).* [Poster presentation]. American Society of Hematology, Orlando, FL, USA. Abstract 5488