Supplementary Data

Tabelecleucel for EBV^{+} PTLD following allogeneic HCT or SOT in a multicenter expanded access protocol

Nikiforow S, Whangbo JS, Reshef R *et al*

This appendix has been provided by the authors to give readers additional information about their work.

Supplemental Table 1. Key Inclusion and Exclusion Criteria

Key Inclusion Criteria
Diagnosis of EBV$^+$ PTLD after allogeneic HCT or SOT
Presence of EBV$^+$ disease (as shown on biopsy or a combination of circulating EBV DNA and radiographic appearance consistent with an EBV$^+$ malignancy, if biopsy is not clinically feasible)
Availability of appropriate HLA partially matched and restricted tabelecleucel cell product
Lack of approved alternative therapies
ECOG performance status ≤4* or Lansky performance status ≥20†
Morphologic remission of underlying disease for patients receiving allogeneic HCT
Adequate organ function per the following: • Absolute neutrophil count ≥500/µL ± cytokine support • Platelets ≥50,000/µL ± transfusion support • Platelets <50,000/µL but ≥20,000/µL ± transfusion support permissible if the patient had not had grade ≥2 bleeding in the preceding 6 months (per NCI-CTCAE v4.03) • Alanine aminotransferase, aspartate aminotransferase, total bilirubin <3 x ULN or ≤5 × ULN if abnormal due to EBV-associated disease involvement of the liver • Creatinine <3 × ULN
Key Exclusion Criteria
Any investigational therapy received ≤4 weeks prior to cycle 1 day 1
Ongoing need for methotrexate or extracorporeal photopheresis; patients receiving corticosteroid therapy >0.5 mg/kg daily were excluded
Need for vasopressor or ventilatory support, unless deemed to be caused by the EBV$^+$ process that tabelecleucel is designed to treat
Antithymocyte globulin, alemtuzumab, or similar T-cell antibody therapy or T-cell therapy received ≤4 weeks prior to cycle 1 day 1

EBV, Epstein-Barr virus; EBV$^+$, Epstein-Barr virus positive; ECOG, Eastern Cooperative Oncology Group; HCT, hematopoietic cell transplant; HLA, human leukocyte antigen; NCI-CTCAE, National Cancer Institute's Common Terminology Criteria for Adverse Events; PTLD, post-transplant lymphoproliferative disease; SOT, solid organ transplant ULN, upper limit of normal.

*For patients aged >16 years.

†For patients aged ≤16 years.

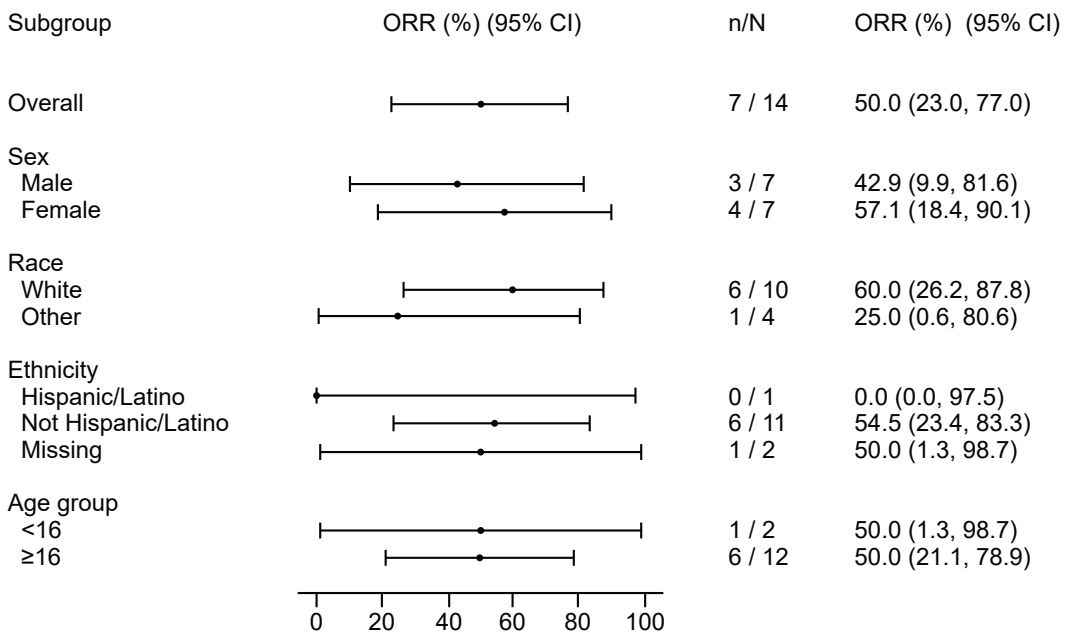
Supplemental Table 2. Definitions of Secondary Efficacy Endpoints

Secondary endpoint	Definition
OS	Time from first dose of tabelecleucel to date of death from any cause
Time to response	Time from the first dose of tabelecleucel to first response (PR or CR)
Duration of response	Measured from the time of initial response until disease progression after the last response or death due to any cause
Progression-free survival	Time from the first dose of tabelecleucel to either progression after the last response or death, whichever occurs first
Durable response rate	Response with a duration >6 months; defined as the proportion of patients with durable response
Time to progression	Time from the first dose of tabelecleucel to progression after the last response

CR, complete response; OS, overall survival; PR, partial response.

Supplemental Figure 1. Forest Plots of Objective Response Rate for the A) HCT (n=14) and B) SOT (n=12) Cohorts

A. HCT cohort (n=14)



B. SOT cohort (n=12)