Background KEYNOTE-048 has demonstrated extended survival benefit of pembrolizumab in 1L R/M HNSCC. This study aims to assess real-world overall survival (rwOS).

Methods A retrospective cohort study was conducted using the Flatiron Health Advanced Head and Neck database. The study cohort included adult R/M HNSCC patients, who initiated 1L pembrolizumab monotherapy (P) or combination with chemotherapy (P+C) between 07/01/19 – 6/30/21 with follow-up until 12/31/21. Patients were excluded if they received prior platinum treatment ≥6 months of 1L pembrolizumab therapy, had other primary cancers before R/M HNSCC diagnosis, or were treated with a clinical study drug. rwOS was assessed using Kaplan-Meier methods, and a multivariate Cox proportional hazard model assessed the effects of age (≥75 vs <75), gender, race, primary tumor site, staging at diagnosis, HPV status, smoking, ECOG PS, and CPS score.

Results A total of 513 patients initiated 1L pembrolizumab therapy, [P (n=337), P+C (n=176)]. The median rwOS was 13.0 months (95%CI 10.0-15.3) for P and 12.8 months (95% CI 9.0-21.1) for P+C (figure 1), consistent with the results of the KEYNOTE-048 trial [P: 11.5 months (10.3 – 13.5) P+C: 13.0 months (10.9 – 14.7)]. Survival rates at 24 months (P: 33.3% (26.2 – 40.6) and P+C: 40.3% (31.3- 49.2) were greater in the real-world compared to KEYNOTE-048. The Cox proportional-hazards model found that older age (≥75 years vs <75) and higher ECOG PS (2+ vs 0-1) were unfavorable predictors of rwOS survival (HR=1.4, 95% CI 1.03-1.90, HR: 1.5, 95%C 1.08-2.1). This analysis further showed no significant differences in rwOS between P and P+C populations.

Conclusions Overall survival among patients treated with pembrolizumab in the real-world were consistent with results from KEYNOTE-048. We found that age and ECOG were predictive of rwOS even after removing the effects of other common predictors, such as race, HPV status, and staging at diagnosis.