

**BLA 125643 Clinical information request 4/28/2017– [Response due COB Wednesday May 3, 2017](#)**

**1. ADTR.xpt (ZUMA1) – post-baseline tumor measurements:**

Per the response to the 4/24/17 IRs, of the 101 patients in the mITT population, 98 had post-baseline tumor burden values. However, for a waterfall plot representing 1 dose of KTE-019 as per the primary analysis plan, if one excludes APERIOD of 2 and selects ADTR.PCHG where MINSPDFL="Y", only 92 patients have results. Thus, 6 patients appear to have missing results.

Of the 9 patients in the mITT period who were retreated, all have postbaseline measures after selecting for period 1; however, in 6 of these patients, the MINSPDFL column is not populated.

- a. Clarify why these 6 patients are missing a flag for minimum post-baseline SPDs for period 1.
- b. Clarify how the minimum post-treatment SPD and associated % change can be obtained for all evaluable patients in the mITT population for period 1 only. If this information is not available in ADTR.xpt, submit a revised ADTR.xpt file that includes this information.
- c. In the dataset requested in part C of the 4/19/2017 information request, containing pre- and post-tumor measurements for the mITT population as assessed by central read, ensure that the MINSPDFL column is populated for all patients having post-baseline tumor measurements in period 1.

**2. ADBASE.xpt (ZUMA1):**

In ADBASE.xpt, 26 patients are categorized as "refractory to first line of therapy" (variable REFSG), but only 2 patients are "primary refractory" (variable REFSG). For efficacy review, since primary refractory disease is standardly the same as refractoriness to first-line therapy, distinguish the terms as used in this study.

**3. ZUMA1 SAP – censoring rules:**

Section 9.5 of the SAP states that, for subjects without documentation of progression prior to initiation of new therapy (excluding autologous SCT), DOR and PFS will be censored at the last disease assessment prior to the initiation of new therapy. For the primary analysis, clarify that these same censoring rules apply to patients who receive subsequent allogeneic, rather than autologous, SCT.