ENESTop 192-week results: Treatment-free remission (TFR) in patients (pts) with chronic myeloid leukemia in chronic phase (CML-CP) after stopping second-line (2L) nilotinib (NIL).

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Abstract Disclosures

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Background: In the ENESTop study (NCT01698905) of TFR in pts with CML-CP who achieved a sustained deep molecular response (MR) with 2L NIL, 57.9% remained in TFR 48 wks after stopping NIL (primary endpoint). Analyses at 144 wks showed durability of TFR. Data from longer follow-up (192 wks) evaluating the maintenance of TFR are reported.**Methods:**Pts treated with ≥2 y NIL after > 4 wks imatinib (≥3 y total) and achieving $MR^{4.5}$ (*BCR-ABL1*^{IS}≤0.0032%) on NIL were eligible. After a 1 y consolidation, pts with no confirmed loss of $MR^{4.5}$ could attempt TFR. NIL was resumed upon loss of major MR (*BCR-ABL1*^{IS}≤0.1%) or confirmed loss of MR^4 (*BCR-ABL1*^{IS}≤0.01%). At the data cut-off (Sep 24 2018), all pts had completed ≥192 wks of TFR, resumed NIL, or discontinued the study.**Results:**By the data cut-off, of 126 pts entering TFR, 56 were ongoing, 59 had resumed NIL, and 11 had discontinued. TFR rate at 192 wks was 46.0% (58/126; 95% CI, 37.1–55.1%); all but 1 of the 58 pts were in $MR^{4.5}$. Only 1/61 pts in TFR at 144 wks lost response by 192 wks (confirmed loss of MR^4); another 2 pts discontinued due to serious AE (polycythemia vera) and pt/guardian decision, respectively. Of 59 pts who resumed NIL, 56 (94.9%) and 55 (93.2%) regained MR^4 and $MR^{4.5}$, respectively. 40/56 pts (71.4%) who regained MR^4 had stable MR^4 at 96 wks (12 discontinued < 96 wks, and 4 remained on study with < 96 wks,

after regaining MR^4); 37/55 pts (67.3%) who regained $MR^{4.5}$ had stable $MR^{4.5}$ at 96 wks (12 discontinued < 96 wks, and 6 remained on study with < 96 wks, after regaining $MR^{4.5}$). There were no disease progressions, deaths due to CML, or new deaths since the 144-wk analysis. The 192 wk treatment-free survival rate was 50.3% (95% CI, 41.2–58.7%). Of 62 pts who remained in TFR for > 144 wks, 11.3%, 53.2%, 21.0%, 14.5% and 3.2% had musculoskeletal pain AEs during consolidation and each subsequent 48 wk period of TFR. Among 59 pts who resumed NIL, most common AEs were hypertension (20.3%) and arthralgia (13.6%); the majority of AEs were grade 1/2.**Conclusions:** Results demonstrate long-term durability and safety of TFR following 2L NIL, with no disease progressions or CML-related deaths, and musculoskeletal pain AEs were transient. Clinical trial information: NCT01698905