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906.OUTCOMES RESEARCH-MYELOID MALIGNANCIES

HJKC3-0006: Outcome of Patients with CML Treated on Second Line or Later Therapy in Routine Clinical Practice

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Background: First-line therapies for chronic-phase (CP) CML include the tyrosine kinase inhibitors(TKIs) dasatinib, nilotinib, bosutinib or imatinib, while ponatinib and asciminib are indicated only in later lines. There are typically high response rates to first-line therapy in CP-CML, with responses to second- and third-line therapy lower and highly dependent on the specific TKI. Our goal in this study is to evaluate the role of ponatinib in the treatment of patients with CML using data from routine clinical practice.

Study design and methods: Data will come from the H. Jean Khoury Cure CML Consortium (HJKC3) CML registry, a North American multi-site research database. Participating sites include the HJKC3 members (curecml.org). Patient inclusion criteria include a diagnosis CML in chronic phase, receiving second line therapy after initial therapy with a second generation TKI (dasatinib, nilotinib, or bosutinib) or receiving third or later line TKI therapy for CML. Participating patients provide consent to collect their data both retrospectively and prospectively for 1 year. Clinical data, including toxicities and complications of TKI therapy, are abstracted from the electronic health record. Participating patients are invited to complete a questionnaire to assess health-related quality of life. Data are managed using REDCap, a secure web application.

Study Objectives: The primary objective is to compare the outcome of patients treated with ponatinib vs. other TKIs as second or later line therapy. Secondary objectives are to evaluate patient-reported outcomes (PRO) for CML treated with second or later line therapy and to determine if there are any patient or disease characteristics that can predict outcomes.

Statistical plan for clinical outcomes: Descriptive statistics will be used to compare baseline demographic characteristics. Categorical variables will be compared using the Chi-square test. Continuous variables will be compared using ANOVA. Overall survival will be calculated from the time of diagnosis to death from any cause and patients will be censored if they were alive at the last follow-up. Survival probabilities will be computed using the Kaplan-Meier method and compared with the log-ranktest. Cox regression analyses will be used to determine significant factors that influence survival, and hazard ratios with 95% confidence intervals will be presented. Event free survival (EFS) and transformation free survival (TFS) will be calculated for patients. For EFS, loss of complete hematologic remission, loss of major cytogenetic response, progression to accelerated or blast phase, or death from any cause at any time will be considered an event. For TFS, transformation to accelerated or blast phase while on therapy or death on study will be considered an event.
Statistical plan for PRO: Different PRO domains will be analyzed separately (for example, depression separate from fatigue, etc.). Descriptive statistics for each PRO measure will be reported and compared to previously published studies. Differences by treatment type will be evaluated.

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