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17TH INTERNATIONAL CONFERENCE FOR  
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WITH CML



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# PRO QOL OF CML PATIENTS ON DIFFERENT TKIs

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Best practice in patient advocacy  
#CMLHZ19





## ***Problem statement and background***

- Tyrosine kinase inhibitors (TKIs) have prolonged the survival of patients with chronic myeloid leukemia (CML). Nowadays, the life expectancy of these patients is approaching that of an age-matched population.
- Since Imatinib, a first generation TKI, was first introduced 20 years ago, several newer more potent TKIs, have been approved for CML treatment. While these drugs are effective in treating CML, they come with the trade-offs of adverse effects. Some are common to all and some are drug-specific.
- As most patients will receive lifelong treatment, quality of life (QOL) is becoming a major concern.
- Yet, QOL under treatment with these drugs has not been directly compared. In this study, we assessed the QOL and symptom burden in a cohort of patients with CML, using a patient reported outcomes (PROs) questionnaire.



## ***Implementation (I)***

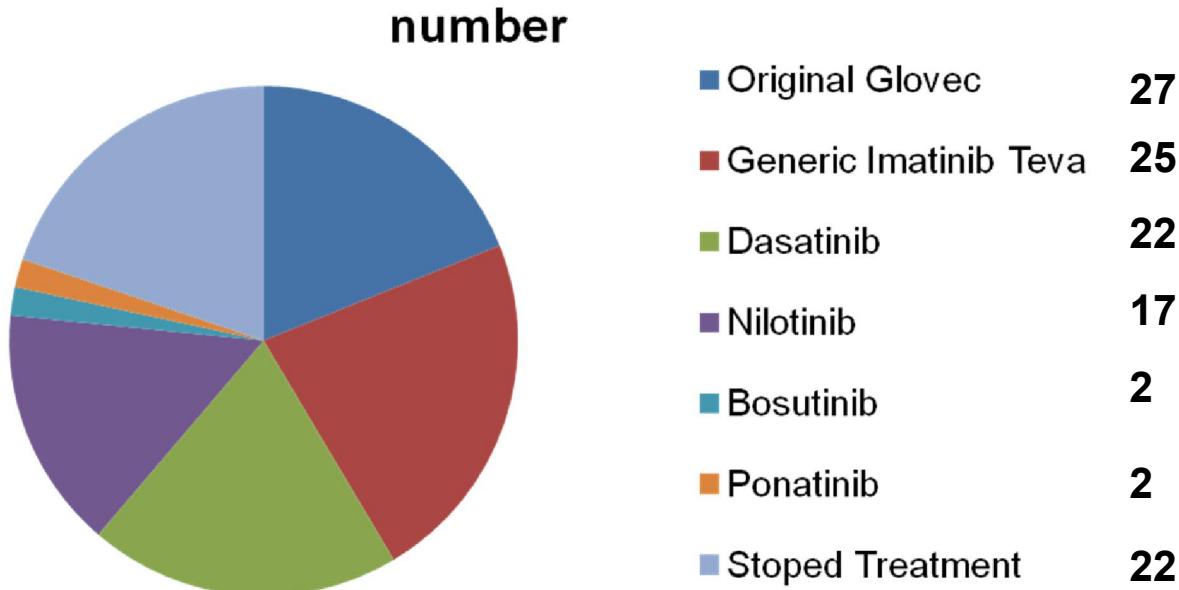
- This study was initiated in collaboration of the Israeli CML Patients Organization with the Hematology department at Rabin medical center.
- Study participants completed the validated European Organization for Research and Treatment of Cancer (EORTC) Q30 items questionnaire and a supplementary EORTC items CML-24 specific questionnaire.
- Additional QOL items were added and in addition, demographics and treatment related information were documented

## Implementation (II)

- Our main challenge was to translate the EORTC CML-24 to Hebrew and get it validated. It is a long and very tedious process which was completed successfully.
- Additional challenges was a quick recruitment and analysis of the study, to get it ready in time for submission of an abstract to EHA.
- Since Hematologists were involved, we needed to get Helsinki approval by the hospital ethics committee.
- Recruitment was done mainly by our members of our PO and in addition the patients in the hospital who are not members of our organization.
- Even though the initial study analysis refers to 127 patients, we have recruited close to 190 patients in just 1 month.
- Data analysis was done by the department statisticians and Hematologists

## ***Result and Impact:***

- Overall, 129 patients completed the questionnaire. The median age of these patients was 56 (range: 25 to 89) years and 67 (60%) were males.
- The median age of patients who received first generation TKI was 67 (range: 32 - 89) years, and 45 years (range: 25 - 82) of patients treated with second or third generation TKIs



## *Result and Impact*

- Patients on first generation TKIs expressed significantly more satisfaction from their quality of life during the preceding week ( $P = 0.006$ ) and during the preceding 3 months ( $P = 0.001$ ).
- Patients on first generation TKIs also reported lesser limitations in daily ( $P = 0.02$ ) and social ( $P = 0.02$ ) activities in the preceding week and also expressed a lesser degree of negative emotions such as anxiety or depression ( $P = 0.006$ ).
- In patients treated with first generation TKIs the symptoms' burden was significantly lower. For example, patients reported of less fatigue ( $P = 0.0021$ ), less pain ( $P = 0.004$ ), less dyspnea ( $P = 0.029$ ) or constipation ( $P = 0.038$ ), lesser degree of impaired body image ( $P = 0.01$ ) during the preceding week if they were treated with first generation TKIs.
- In a linear regression model, only the type of drug (first generation vs. newer generations) predicted global QOL ( $P = 0.006$ ) whereas age and gender did not.



## ***Conclusion and recommendation:***

- The reported QOL of patients with CP-CML who receive first generation TKI is superior to that of patients receiving newer generation TKIs.
- CML AN SC will review the possibility to expand this interesting study into a global multi countries and languages study.
- If the results hold up in a large global study, they might influence patients and HCP decision in choosing a TKI

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