How are we treating our chronic phase chronic myeloid leukemia (CML-CP) patients? An experts' panel perspective from Brazilian healthcare system on treatment access

HSD80

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INTRODUCTION

- Chronic myeloid leukemia (CML) is a hematological neoplasm that, if properly treated in initial stages (chronic phase; CP), progression to advanced phases can be significantly delayed or avoided.[1]
- Treatment with tyrosine kinase inhibitors (TKI) have changed the disease course over time. Although treatment with 1st and 2nd generation TKIs as first- (1L) and second-line (2L) treatments are established and reimbursed in Brazil, in later lines, the availability of effective TKIs is limited and eligibility to hematopoietic stem cell transplantation (HSCT) is rare. [2,3]
- The aim of this study was to analyze therapies available for CML-CP patients in both Brazilian Public and Private Healthcare Systems, the current clinical practices and unmet needs of patients in third-line treatment.

METHODS

- An expert panel was implemented to seek consensus with 14 experienced hematologists from major treatment centers across Brazil.
- Among the participants, average experience in clinical practice was 23 years all were working at university hospitals or training and research hospitals at the time of the panel.
- A series of advisory boards was conducted to capture quantitative and qualitative data about their opinion and current practices in CML-CP treatment..
- During the advisory boards, hematologists were requested individually to share about their experience, local epidemiology, investigation and follow-up routine (exams solicitation, access, and frequency) and rationale for therapy choice (first-, second- and third-line choices, duration, and reasons for change).
- The final section of the boards was comprised of discussions to reach consensus among participants.
- The collected data was stored and analyzed (descriptive statistics) using Microsoft Excel 365.

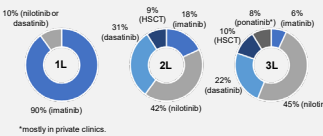
Disclosures

- CP, KP, RC and NH received fees for participating in the advisory boards.
- DdO and DK are employees of Novartis.

RESULTS

- Expert panel data covered 2,157 CML-CP patients' population (82% from the Public Healthcare System).
- All the experts were currently working in hospitals; 78,5% were involved in clinical practice, education and research (working in hospitals, current clinical practice and acting as professors or had academic activities).
- Results demonstrated that 1,510 (70%) patients were receiving first-line treatment, predominantly imatinib (90%). The reported average time until treatment failure was 18 months. Resistance (44%) and intolerance (25%) were the main causes of progression to a second-line treatment.
- 421 patients (20%) were receiving second-line treatment, mostly nilotinib (42%) and dasatinib (31%). 18% of the patients continued with imatinib or were using ponatinib (private clinics).
- The reported average time until progression during second-line treatment was 18 months. Resistance (47.5%) and intolerance (23.7%) were also the main causes of treatment line progression. Other reasons included disease progression and lack of treatment compliance. Approximately 9% of patients in second-line treatment were eligible to hematopoietic stem cell transplantation (HSCT).
- 216 (10%) of patients were in third-line treatment, mainly nilotinib (50%) and dasatinib (20%), and the reported average time until failure was12 months.
- Ponatinib was available for only 20 patients - all in the Private Healthcare System -, bosutinib was not available, and asciminib was available exclusively through clinical research protocol (without regulatory approval at the time the panels were conducted). HSCT was indicated for 10% of third-line patients.

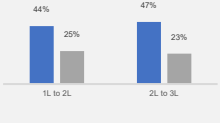
Figure 1. CML-CP therapy use reported by treatment line



*mostly in private clinics.

Figure 2. Reported average time until treatment failure per line of treatment

Figure 3. Causes of treatment line failure



DISCUSSION AND CONCLUSION

- In conclusion, the expert panel evaluated the CML-CP scenario in Brazil as quite variable according to the type of care (public or private), number of patients treated (reference center *versus* individual clinics) and regional peculiarities.
- For first-line treatment, the experts concluded that most CML-CP patients respond adequately to 1st or 2nd generation TKIs.
- In second-line, efficacy is established and the experts express concerns about the adverse events of available drugs and limitations of mutation testing.
- For third-line, the overall feeling is insecurity and laxity, highlighting the lack of standardization and experience with third-line drugs as major limitations of care.

References

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