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
- New options are emerging for patients who progressed after two prior TKIs; hence, understanding the current cost of treatment is key to drive appropriate decision making in the Brazilian Private Healthcare System.
- The objective of this study is to analyze the financial burden of CML-CP treatment for patients who failed two or more prior TKIs from the Brazilian Private Healthcare System perspective.

METHODS

- This study estimated the financial burden of 3L and 4L of CML from the Brazilian Private Healthcare System perspective, including drug, monitoring and HSCT costs.
- Target population was obtained through an epidemiological approach considering adult patients diagnosed with CML-CP, who were previously treated with at least 2 TKIs. From the Brazilian population covered by private insurance, a prevalence of 5 per 100,000 inhabitants4 was considered of which 90% of patients were in the CP5. It was assumed that 30% of patients would progress from 1L to 2L and 50% to 3L.
- Treatment switch from 3L to 4L was estimated as the percentage of failure (75%) to 2nd generation TKI, as reported by Akard et al. (2013)6.
- It is important to highlight that this model only analyzed the treatment costs and did not assess the cost-effectiveness of the interventions.
- CML patients in later lines of therapy demonstrate relevant disease management-related expenditures. Among the TKIs, ponatinib use in 4L demonstrated the highest treatment cost. Although HSCT is associated with elevated costs, its eligibility is limited to a small patient population.
- Significant costs regarding failure of second line treatment highlight the medical unmet need for new effective technologies in CML treatment landscape, especially in later lines.

RESULTS

- A total of 375 patients in CML-CP who previously received two TKIs were estimated from the Brazilian Private Healthcare System in 2023.
- In 3L, only 6.08% of patients were eligible for HSCT (26,908,000 BRL [5,307,298 USD]) and 6.129 BRL (1,209 USD) for 3L and 4L, respectively.
- The total cost of 3L and 4L was 127,143,335 BRL (22,077,581 USD) and 137,045,192 BRL (27,030,610 USD), respectively.

<table>
<thead>
<tr>
<th>Parameter</th>
<th>Cost</th>
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<tbody>
<tr>
<td>Drugs</td>
<td></td>
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<tr>
<td>Nilotinib</td>
<td>31,075 (6,130)</td>
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<tr>
<td>Dasatinib</td>
<td>16,005 (3,157)</td>
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<tr>
<td>Ponatinib</td>
<td>10,954 (2,161)</td>
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<tr>
<td>HSCT</td>
<td>22,487,400 (4,435,385 USD)</td>
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<tr>
<td>Follow-up costs</td>
<td>8,895 BRL (1,754 USD)</td>
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</tbody>
</table>

CONCLUSION

- CML patients in later lines of therapy demonstrate relevant disease management-related expenditures. Among the TKIs, ponatinib use in 4L demonstrated the highest treatment cost. Although HSCT is associated with elevated costs, its eligibility is limited to a small patient population.
- Significant costs regarding failure of second line treatment highlight the medical unmet need for new effective technologies in CML treatment landscape, especially in later lines.
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References