

# Hematopoietic cell transplantation in pediatric chronic myeloid leukemia

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## ABSTRACT

Chronic myeloid leukemia (CML) is a clonal myeloproliferative disorder primarily linked to the *BCR-ABL* fusion gene, resulting from the Philadelphia chromosome translocation. While rare in pediatric populations, CML in children presents unique biological and clinical features, often diagnosed incidentally through leukocytosis. Symptoms may include fatigue, weight loss, and splenomegaly, with advanced cases exhibiting signs of bleeding and infections. The management of pediatric CML has evolved significantly with the introduction of tyrosine kinase inhibitors (TKIs), in which imatinib remains the first-line treatment despite potential long-term toxicities. Second-generation TKIs such as dasatinib and nilotinib are alternatives for treatment resistance. Asciminib, a third-generation inhibitor, shows promise for cases resistant to other TKIs, although pediatric data are still emerging. Allogeneic hematopoietic cell transplantation (HCT) is crucial for high-risk pediatric CML cases, particularly in the blastic phase or in instances of TKI resistance. The choice of conditioning regimens, whether myeloablative or reduced-intensity, is vital, with busulfan-based regimens preferred due to lower late effects. Post-transplant, the use of TKIs can be beneficial, with molecular monitoring guiding treatment decisions. Overall, individualization of therapy considering disease phase, molecular profile, and donor availability is essential for optimizing outcomes in pediatric CML management. Close follow-up strategies involving regular molecular assessments and tailored therapeutic approaches post-HCT are critical for improving patient survival rates and managing relapses.

**Keywords:** Pediatrics. Leukemia, Myeloid. Hematopoietic Stem Cell Transplantation.

## INTRODUCTION

Chronic myeloid leukemia (CML) is a clonal myeloproliferative disorder characterized by the t(9;22) translocation (Philadelphia chromosome), generating the BCR-ABL1 fusion gene. The resulting oncoprotein is a constitutively active tyrosine kinase that drives disease pathogenesis. Although pediatric CML is rare, it exhibits distinct biological and clinical characteristics compared to adult cases.

In children, CML often presents insidiously and is frequently diagnosed incidentally via leukocytosis. Symptoms may include fatigue, weight loss, bone pain, and abdominal discomfort from splenomegaly. Bleeding, infections, or leukostasis signs (*e.g.*, headache, visual changes) may occur in advanced stages. Compared to adults, children tend to have higher white blood cell counts, larger spleens, more advanced-phase disease, and a distinct BCR-ABL1 profile with more driver mutations.

Adult CML risk scores (Sokal, EURO, EUTOS) lack consistent predictive value in children. The ELTS score may help estimate progression-free survival but not overall survival. It uses age, spleen size, blasts, and platelets to stratify risk<sup>1</sup>.

## USE OF TYROSINE KINASE INHIBITORS AND ASCIMINIB IN CHILDREN AND ADOLESCENTS

Tyrosine kinase inhibitors (TKIs) have transformed CML management in children. Imatinib, a first-line agent for pediatric chronic myeloid leukemia chronic phase (CML-CP), is effective but associated with lower deep molecular response rates and potential long-term toxicities, including growth impairment, metabolic disturbances, and other late effects due to the prolonged duration of treatment<sup>2,3</sup>.

Second-generation TKIs (dasatinib, nilotinib) are used in cases of imatinib resistance or intolerance. Dasatinib penetrates the central nervous system, while nilotinib requires metabolic monitoring due to cardiovascular risks<sup>3,4</sup>.

Asciminib, a third-generation allosteric inhibitor targeting the ABL myristoyl pocket, has shown efficacy in resistant adult CML, including T315I mutations. Pediatric data are limited, but early studies are ongoing and suggest promise in TKI-resistant/intolerant cases (Table 1).

Table 1 summarizes key pediatric-relevant TKI properties, including approvals, resistance profiles, toxicities, and current trial status<sup>3-8</sup>.

**Table 1.** Comparison of tyrosine kinase inhibitors.

Therapy	FDA approved in pediatrics	Generation	Dosing	ABL mutation associated with resistance	Toxicities	Other
Imatinib	Yes	First	260–340 mg/m <sup>2</sup> /dose once daily With or without food Maximum dose: 400 mg/day	Too many to list	Muscle cramps Edema Diarrhea	
Dasatinib	Yes	Second	60 mg/mg/m <sup>2</sup> /dose once daily With or without food	T315I/A, F317L/V/ I/C, V299L	Pleural/pericardial effusions; pulmonary hypertension; GI bleeding; QTc prolongation	Cross the blood-brain barrier
Nilotinib	Yes	Second	230 mg/m <sup>2</sup> /dose No food 2 hours prior and 1 hour after intake	T315I, Y253H, E255K/V, F359V/C/I	QTc prolongation; arterial occlusion; metabolic changes (glucose/lipids)	
Bosutinib	No	Second	290–330 mg/m <sup>2</sup> /day <sup>4</sup> daily With food ClinicalTrials.gov (NCT 04258943)	T315I, V299L, G250E, F317L	Diarrhea; hepatic enzyme increase	
Ponatinib	No	Third	Median dose <sup>5,6</sup> : 16.9 to 21.4 mg/m <sup>2</sup> Daily; With or without food Pediatric phase I/II trials with or without chemotherapy: NCT 04501614 and NCT03934372	Rare	Arterial and venous thrombosis; pancreatitis; elevated alanine and aspartate aminotransferases; hypertension; polymorphic erythema; myelosuppression	Effective with T315I mutation
Asciminib <sup>7</sup>	No	Third Targets the ABL myristoyl pocket (STAMP), unlike ATP- competitive TKIs	In adults: 80 mg once daily or 40 mg BID for patients with non-T315I mutated CML-CP and 200 mg BID for T315I-mutated CML-CP In pediatric CML <sup>8</sup> : part 1: dose determining cohort: minitab of 1.3 mg/kg with food. Part 2: expansion cohort, 40 mg BID Part 3: expansion cohort, 80 mg QID	Rare	Myelosuppression, pancreas enzyme elevation, pancreatitis; hypertension; fatigue; headache	Effective with T315I mutation

FDA: Food and Drug Administration; GI: gastrointestinal tract; TKI: tyrosine kinase inhibitor; BID: twice daily; CML-CP: chronic myeloid leukemia in chronic phase; QID: four times daily; ABL: abelson leukemia gene; ATP: adenosine triphosphate; QTc: corrected QT interval. Source: modified from Ford et al. and Hijjiya et al.<sup>2,3</sup>.

In Brazil, imatinib is the first-line treatment for pediatric CML-CP due to its efficacy and availability in the public system. Monitoring follows European LeukemiaNet guidelines, with BCR-ABL1 polymerase chain reaction (PCR) every three months. Dasatinib and nilotinib are alternatives in cases of imatinib resistance or intolerance<sup>3,4</sup>. Challenges include delayed diagnosis, limited PCR access, and restricted availability of newer TKIs. Multicenter initiatives aim to improve access and outcomes.

## HEMATOPOIETIC CELL TRANSPLANTATION INDICATIONS

Allo-HCT is indicated in children with CML under specific high-risk conditions: blastic phase, atypical CML, TKI-refractory or relapsed disease, and presence of mutations like T315I.

Conditioning: both myeloablative conditioning (MAC) and reduced intensity conditioning (RIC) are used. RIC may be suitable for CML-CP, offering similar overall survival with less toxicity<sup>9</sup>.

Donor: matched sibling donors are preferred; matched unrelated donors are viable alternatives with improving outcomes<sup>10,11</sup>.

Stem cell source: bone marrow is preferred due to lower chronic graft-versus-host disease (GVHD); peripheral blood stem cells (PBSCs) have higher GVHD risk, and cord blood (CB) has lower engraftment success<sup>9,12</sup>.

In blastic phase, transplantation should occur in remission when possible<sup>13</sup>.

In atypical CML, allo-HCT is the only curative option<sup>14</sup>.

In relapsed/refractory disease, transplantation is considered after assessment of response and risk<sup>15</sup>.

Individualized decisions based on disease phase, molecular profile, and donor availability are essential to optimize outcomes.

## CONDITIONING REGIMEN AND GRAFT-VERSUS-HOST DISEASE PROPHYLAXIS

Allo-HCT remains the only curative option for pediatric CML, especially in advanced or refractory cases, as the response to TKIs may be temporary and little is known about the long-term effects of the prolonged use of these medications<sup>16,17</sup>. Moreover, the success of TKI treatment also depends on the patient's adherence<sup>18</sup>. First-line HCT has demonstrated results equivalent to TKI therapy<sup>19</sup>. Outcomes mainly depend on the disease phase at transplant—blastic phase being associated with poor prognosis, particularly if not reverted to chronic phase beforehand<sup>16,18</sup>.

MAC regimens, especially busulfan/cyclophosphamide (BU/CY), are preferred over total body irradiation-based regimens in children due to lower risk of late effects<sup>11,20,21</sup>. Intravenous BU is associated with lower relapse risk<sup>20</sup> (Table 2).

**Table 2.** Types of conditioning for matched related and unrelated donors.

Myeloablative	Reduced intensity conditioning
IV Busulfan (dose according to body weight* or adjustment based on pharmacokinetic studies, if available): D-8 to D-5 + Cyclophosphamide 60 mg/kg/day + Mesna (150% of CY dose): D-2 and D-1 + rATG 5–6 mg/kg/total dose D-3 to D-1 (if unrelated donor) GVHD prophylaxis: Cyclosporine + MTX (D+1, D+3, D+6)	Fludarabine 30 mg/m <sup>2</sup> /day: D-10 to D-5 + IV Busulfan (dose according to body weight* or adjustment based on pharmacokinetic studies, if available): D-6 and D-5 + rATG 5–6 mg/kg/total dose D-4 to D-1 GVHD prophylaxis: Cyclosporine + MTX (D+1, D+3, D+6)
	Fludarabine 40 mg/m <sup>2</sup> /day: D-7 to D-4 + Thiotepa 5 mg/kg BID D-3 + Melphalan 140 mg/m <sup>2</sup> D-2 + rATG D-3 to D-1 (for all patients – MSD or MUD) GVHD prophylaxis: Cyclosporine + MMF (from D+1 until D+30)

\*IV busulfan daily dose = < 9 kg: 4 mg/kg; 9 to < 16 kg: 4.8 mg/kg; 16–23 kg: 4.4 mg/kg; > 23 to 34 kg: 3.8 mg/kg; > 34 kg: 3.2 mg/kg; rATG: rabbit anti-thymocyte globulin; GVHD: graft-versus-host disease; MTX: methotrexate; MSD: matched sibling donor; MUD: matched unrelated donor; MMF: mycophenolate mofetil. Source: Zhao et al.<sup>18</sup>, Copelan et al.<sup>20</sup>, Suttorp et al.<sup>21</sup> and Pichler et al.<sup>22</sup>.

Total body irradiation remains an option, but it should be avoided in young patients when possible, due to its long-term toxicity (e.g., growth impairment, secondary malignancies)<sup>21</sup>.

RIC regimens have shown promising results in pediatric CML-CP, with lower transplant related mortality (TRM) and good overall survival. Examples include: fludarabine + BU + post-HCT imatinib<sup>18</sup> and fludarabine, thiotepa, and melphalan + ATG<sup>22</sup> (Table 2).

In blastic phase, MAC is still recommended after achieving remission.

Data on haploidentical HSCT for pediatric CML remain limited, but the approach may be considered in selected cases when no matched donor is available<sup>18,23</sup>.

Individualization of conditioning intensity should balance disease status, age, comorbidities, and long-term toxicity.

### TYROSINE KINASE INHIBITORS USE AFTER HEMATOPOIETIC CELL TRANSPLANTATION

TKIs can be used after HCT either prophylactically (after engraftment and hematologic recovery) or preemptively, based on rising BCR-ABL1 transcript levels<sup>16</sup>. Retrospective and prospective studies have shown a survival benefit with post-transplant TKI use<sup>16</sup>. When close molecular monitoring is feasible, a preemptive strategy may be preferred<sup>16</sup>.

The International BFM study demonstrated excellent five-year survival (95%) using molecular monitoring to guide TKI reintroduction and donor lymphocyte infusion (DLI). The 2025 International Pediatric CML Expert Panel recommends restarting the same pre-HCT TKI in case of confirmed major molecular response (MMR) loss, and considering immunosuppression taper or DLI based on risk-benefit analysis<sup>22,24</sup>.

For patients not undergoing HCT, TKI discontinuation may be attempted after  $\geq$  three years of therapy and  $\geq$  two years of sustained MR4<sup>3</sup>. Post-HCT, TKIs should be continued for at least two years after achieving deep molecular remission<sup>25</sup>.

Additional relapse prevention strategies include disease control before transplant, close BCR-ABL1 monitoring post-HCT, optimizing conditioning regimens, minimizing immunosuppression, and prophylactic DLI to enhance graft *versus* leukemia (GVL)<sup>19,26</sup>.

### POST-HEMATOPOIETIC CELL TRANSPLANTATION FOLLOW-UP

A prospective multicenter trial of the International BFM Study Group included intense post-transplant monitoring of molecular response (monthly in the first and second years post-HSCT, or sooner, if BCR:ABL1 is detected)<sup>22</sup>. This strategy guided TKI retreatment in the first year and DLI in the second-year post-transplant. DLI were postponed to the second year after HCT to mitigate the risk for GVHD<sup>22,27</sup>.

The 2025 National Comprehensive Cancer Network guidelines suggests post-HCT follow-up with quantitative PCR (peripheral blood) every three months for two years, then every three to six months<sup>28</sup>. If negative, consider TKI therapy for at least one year in patients with prior accelerated phase chronic myeloid leukemia (AP-CML) or blast phase chronic myeloid leukemia (BP-CML). If positive, restart TKI treatment (selection of TKI is based on prior therapy, BCR::ABL1 mutation profile, and post-HCT morbidities) and DLI<sup>28</sup>.

The BCR::ABL1 kinase domain mutation analysis must be carried out when there is any sign of loss of complete cytogenetic response (CCyR) or its molecular response correlate or loss of MMR<sup>28</sup>.

### CONFLICT OF INTEREST

Nothing to declare.

## DATA AVAILABILITY STATEMENT

Data sharing is not applicable.

## AUTHORS' CONTRIBUTIONS

**Substantive scientific and intellectual contributions to the study:** Gouveia RV, Michalowski MB, Klinger PHS, Domingues LS, Correa ACR, Caleffi MF, Macedo AV. **Conception and design:** Gouveia RV, Michalowski MB, Klinger PHS, Domingues LS, Correa ACR, Caleffi MF, Macedo AV. **Analysis and interpretation of data:** Gouveia RV, Michalowski MB, Klinger PHS, Domingues LS, Correa ACR, Caleffi MF, Macedo AV. **Manuscript writing:** Gouveia RV, Michalowski MB, Klinger PHS, Domingues LS, Correa ACR, Caleffi MF, Macedo AV. **Final approval:** Gouveia RV, Michalowski MB, Klinger PHS.

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## DECLARATION OF USE OF ARTIFICIAL INTELLIGENCE TOOLS

Not applicable.

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