Hematopoietic cell transplantation in pediatric chronic myeloid leukemia

Roseane Vasconcelos Gouveia[1,2*], Mariana Bohns Michalowski[3], Paulo Henrique dos Santos Klinger[4], Luciana dos Santos Domingues[2], Ana Carolina Ribeiro Correa[2], Mariane Farherr Caleffi[5], Antonio Vaz de Macedo[6,7]

1. Hospital Samaritano de São Paulo – São Paulo (SP), Brazil.
2. Universidade Federal de São Paulo – Grupo de Apoio ao Adolescente e à Criança com Câncer – São Paulo (SP), Brazil.
3. Universidade Federal do Rio Grande do Sul – Hospital de Clínicas de Porto Alegre – Porto Alegre (RS), Brazil.
4. Hospital Santa Marcelina – Associação para Criança e Adolescente com Câncer – São Paulo (SP), Brazil.
5. Hospital Erastinho – Curitiba (PR), Brazil.
6. Hospital da Polícia Militar – Belo Horizonte (MG), Brazil.
7. Hospital Luxemburgo – Belo Horizonte (MG), Brazil.

***Corresponding author:** roseanegouveia@yahoo.com

Section editor: Fernando Barroso Duarte

Received: Sept. 15, 2025 • **Accepted:** Jan. 5, 2026

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[1].

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 myeloide 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[2,3].

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[3,4].

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[3–8].

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^2/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^2/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^2/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^2/day^4 daily With food ClinicalTrials.gov (NCT 04258943)	T315I, V299L, G250E, F317L	Diarrhea; hepatic enzyme increase	
Ponatinib	No	Third	Median dose[5,6]: 16.9 to 21.4 mg/m^2 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[7]	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[8]: part 1: dose determining cohort: minitablet 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 Hijiya et al.[2,3].

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[3,4]. 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[9].

Donor: matched sibling donors are preferred; matched unrelated donors are viable alternatives with improving outcomes[10,11].

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[9,12].

In blastic phase, transplantation should occur in remission when possible[13].

In atypical CML, allo-HCT is the only curative option[14].

In relapsed/refractory disease, transplantation is considered after assessment of response and risk[15].

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[16,17]. Moreover, the success of TKI treatment also depends on the patient's adherence[18]. First-line HCT has demonstrated results equivalent to TKI therapy[19]. Outcomes mainly depend on the disease phase at transplant—blastic phase being associated with poor prognosis, particularly if not reverted to chronic phase beforehand[16,18].

MAC regimens, especially busulfan/cyclophosphamide (BU/CY), are preferred over total body irradiation-based regimens in children due to lower risk of late effects[11,20,21]. Intravenous BU is associated with lower relapse risk[20] (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^2/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^2/day: D-7 to D-4 + Thiotepa 5 mg/kg BID D-3 + Melphalan 140 mg/m^2 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.[18], Copelan et al.[20], Suttorp et al.[21] and Pichler et al.[22].

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)[21].

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[18] and fludarabine, thiotepa, and melphalan + ATG[22] (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[18,23].

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[16]. Retrospective and prospective studies have shown a survival benefit with post-transplant TKI use[16]. When close molecular monitoring is feasible, a preemptive strategy may be preferred[16].

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[22,24].

For patients not undergoing HCT, TKI discontinuation may be attempted after ≥ three years of therapy and ≥ two years of sustained MR4[3]. Post-HCT, TKIs should be continued for at least two years after achieving deep molecular remission[25].

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* leucemia (GVL)[19,26].

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)[22]. 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[22,27].

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[28]. 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[28].

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[28].

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.

FUNDING

Not applicable.

DECLARATION OF USE OF ARTIFICIAL INTELLIGENCE TOOLS

Not applicable.

ACKNOWLEDGEMENTS

Not applicable.

REFERENCES

1. Millot F, Guilhot J, Suttorp M, Güneş AM, Sedlacek P, De Bont E, Li CK, Kalwak K, Lausen B, Culic S, Dworzak M, Kaiserova E, De Moerloose B, Roula F, Biondi A, Baruchel A. Prognostic discrimination based on the EUTOS long-term survival score within the International Registry for Chronic Myeloid Leukemia in children and adolescents. Haematologica. 2017;102(10):1704–8. https://doi.org/10.3324/haematol.2017.170035
2. Ford M, Mauro M, Aftandilian C, Sakamoto KM, Hijiya N. Management of chronic myeloid leukemia in children and young adult. Curr Hematol Malig Rep. 2022;17:121–6. https://doi.org/10.1007/s11899-022-00673-5
3. Hijiya N, Suttorp M. How I treat chronic myeloid leukemia in children and adolescents. Blood. 2019; 133(22):2374–84. https://doi.org/10.1182/blood.2018882233
4. Inoue A, Imamura CK, Shimada H, Katayama D, Urabe K, Suzuki R, Takitani K, Ashida A. Pharmacokinetics, efficacy and safety of bosutinib in a pediatric with chronic myeloid leukemia. J Pediatr Pharmacol Ther. 2020;25(8):742–5. https://doi.org/10.5863/1551-6776-25.8.742
5. Kodama Y, Sato A, Kato K, Sakaguchi H, Kato M, Kawasaki H, Hiramatsu H, Kato I, Taga T, Shimada H. Ponatinib in pediatric patients with Philadelphia chromosome-positive leukemia: a retrospective survey of the Japan children's Cancer Group. Int J Hematol. 2022;116(1):131–8. https://doi.org/10.1007/s12185-022-03329-5
6. Millot F, Suttorp M, Versluys AB, Kalwak K, Nelken B, Ducassou S, Bertrand Y, Baruchel A. Ponatinib in childhood Philadelphia chromosome-positive leukaemias: an international registry of childhood chronic myeloid leukaemia study. Eur J Cancer. 2020;136:107–12 https://doi.org/10.1016/j.ejca.2020.05.020
7. Hijiya N, Mauro MJ. Asciminib in the treatment of Philadelphia chromosome-positive cronic myeloid leukemia: Focus on patient selection and outcomes. Cancer Manag Res. 2023;15:873-91. https://doi.org/10.2147/CMAR.S353374

8. Hijiya N, Kapoor S, Hoch M, Descamps L, Dhamal V, Cardoso APT. ASC4KIDS: A multicenter, open-label, phase Ib/II study to determine the dose and safety of Asciminib in pediatric patients with chronic myeloid leukemia in the chronic phase. The 65th ASH Annual Meeting Abstracts. Blood. 2023;142(Suppl.1):6387. https://doi.org/10.1182/blood-2023-185535

9. Shimada H, Tanizawa A, Kondo T, Nagamura-Inoue T, Yasui M, Tojo A, Muramatsu H, Eto T, Doki N, Tanaka M, Sato M, Noguchi M, Uchida N, Takahashi Y, Sakata N, Ichinohe T, Hashii Y, Kato K, Atsuta Y, Ohashi K; Pediatric and Adult CML/MPN Working Groups of the Japanese Society for Transplantation and Cellular Therapy. Prognostic factors for outcomes of allogeneic HSCT for children and adolescents/young adults with CML in the TKI era. Transplant Cell Ther. 2022;28(7):376–89. https://doi.org/10.1016/j.jtct.2022.04.011

10. Millot F, Esperou H, Bordigoni P, Dalle JH, Michallet M, Michel G, Bourhis JL, Kuenz M, Blaise D, Garban F, Plouvier E, Rome A, Guilhot J, Guilhot F. Allogeneic bone marrow transplantation for chronic myeloid leukemia in childhood: a report from the Société Française de Greffe de Moelle et de Thérapie Cellulaire (SFGM-TC). Bone Marrow Transplantation. 2003;32(10):993–9. https://doi.org/10.1038/sj.bmt.1704255

11. Cwynarski K, Roberts IA, Iacobelli S, van Biezen A, Brand R, Devergie A, Vossen JM, Aljurf M, Arcese W, Locatelli F, Dini G, Niethammer D, Niederwieser D, Apperley JF; Paediatric and Chronic Leukaemia Working Parties of the European Group for Blood and Marrow Transplantation. Stem cell transplantation for chronic myeloid leukemia in children. Blood. 2003;102(4):1224–31. https://doi.org/10.1182/blood-2002-12-3637

12. Hafez HA, Abdallah A, Hammad M, Hamdy N, Yassin D, Salem S, Hassanain O, Elhalaby L, Elhaddad A. Outcomes of allogenic hematopoietic cell transplantation for childhood chronic myeloid leukemia: single-center experience. Pediatr Transplant. 2020;24(4):e13664. https://doi.org/10.1111/petr.13664

13. Ishida H, Shimada H, Tanizawa A, Shimazu Y, Tachibana T, Doki N, Ara T, Matsuo Y, Nara M, Toubai T, Ino K, Nakamae H, Kato K, Kato K, Sato A, Hino M, Matsumoto K, Atsuta Y, Yasui M, Nagamura-Inoue T. Allogeneic stem cell transplantation for children and adolescents/young adults with de novo blastic phase chronic myeloid leukemia in the tyrosine kinase inhibitor era. Am J Hematol. 2023;98(8):E200–3. https://doi.org/10.1002/ajh.26959

14. Onida F, de Wreede LC, van Biezen A, Eikema DJ, Byrne JL, Iori AP, Schots R, Jungova A, Schetelig J, Finke J, Veelken H, Johansson JE, Craddock C, Stelljes M, Theobald M, Holler E, Schanz U, Schaap N, Bittenbring J, Olavarria E, Chalandon Y, Kröger N. Allogeneic stem cell transplantation in patients with atypical chronic myeloid leukaemia: a retrospective study from the Chronic Malignancies Working Party of the European Society for Blood and Marrow Transplantation. Br J Haematol. 2017;177(5):759–65. https://doi.org/10.1111/bjh.14619

15. Fraint E, Abdel-Azim H, Bhatt NS, Broglie L, Chattha A, Kohorst M, Ktena YP, Lee MA, Long S, Qayed M, Sharma A, Stefanski H, Vatsayan A, Wray L, Hamadani M, Carpenter PA. Evaluation of children with malignancies for blood and marrow transplantation: a report from the ASTCT Committee on Practice Guidelines. Transplant Cell Ther. 2023;29(5):293–301. https://doi.org/10.1016/j.jtct.2023.02.003

16. Sembill S, Ampatzidou M, Chaudhury S, Dworzak M, Kalwak K, Karow A, Kiani A, Krumbholz M, Luesink M, Naumann-Bartsch N, De Moerloose B, Osborn M, Schultz KR, Sedlacek P, Giona F, Zwaan CM, Shimada H, Versluijs B, Millot F, Hijiya N, Suttorp M, Metzler M. Management of children and adolescents with chronic myeloid leukemia in blast phase: International pediatric CML expert panel recommendations. Leukemia. 2023;37(3):505–17. https://doi.org/10.1038/s41375-023-01822-2

17. Athale U, Hijiya N, Patterson BC, Bergsagel J, Andolina JR, Bittencourt H, Schultz KR, Burke MJ, Redell MS, Kolb EA, Johnston DL. Management of chronic myeloid leukemia in children and adolescents: Recommendations from the Children's Oncology Group CML Working Group. Pediatr Blood Cancer. 2019;66(9):e27827. https://doi.org/10.1002/pbc.27827

18. Zhao Y, Wang J, Luo Y, Shi J, Zheng W, Tan Y, Cai Z, Huang H. Reduced-intensity allogeneic hematopoietic stem cell transplantation combined with imatinib has comparable event-free survival and overall survival to long-term imatinib treatment in young patients with chronic myeloid leukemia. Ann Hematol. 2017;96(8):1353–60. https://doi.org/10.1007/s00277-017-3021-y

19. Craddock CF. We do still transplant CML, don't we? Hematology Am Soc Hematol Educ Program. 2018;2018(1):177–84. https://doi.org/10.1182/asheducation-2018.1.177

20. Copelan EA, Avalos BR, Ahn KW, Zhu X, Gale RP, Grunwald MR, Hamadani M, Hamilton BK, Hale GA, Marks DI, Waller EK, Savani BN, Costa LJ, Ramanathan M, Cahn JY, Khoury HJ, Weisdorf DJ, Inamoto Y, Kamble RT, Schouten HC, Wirk B, Litzow MR, Aljurf MD, van Besien KW, Ustun C, Bolwell BJ, Bredeson CN, Fasan O, Ghosh N, Horowitz MM, Arora M, Szer J, Loren AW, Alyea EP, Cortes J, Maziarz RT, Kalaycio ME, Saber W. Comparison of outcomes of allogeneic transplantation for chronic myeloid leukemia with cyclophosphamide in combination with intravenous busulfan, oral busulfan, or total body irradiation. Biol Blood Marrow Transplant. 2015;21(3):552–8. https://doi.org/10.1016/j.bbmt.2014.12.010

21. Suttorp M, Claviez A, Bader P, Peters C, Gadner H, Ebell W, Dilloo D, Kremens B, Kabisch H, Führer M, Zintl F, Göbel U, Klingebiel T. Allogeneic stem cell transplantation for pediatric and adolescent patients with CML: results from the prospective trial CML-paed I. Klin Padiatr. 2009;221(6):351–7. https://doi.org/10.1055/s-0029-1239529

22. Pichler H, Sedlacek P, Meisel R, Beier R, Faraci M, Kalwak K, Ifversen M, Müller I, Stein J, Vettenranta K, Kropshofer G, Kolenova A, Karlhuber S, Glogova E, Poetschger U, Peters C, Suttorp M, Matthes-Leodolter S, Balduzzi A. Haematopoietic stem cell transplantation after reduced intensity conditioning in children and adolescents with chronic myeloid leukaemia: A prospective multicentre trial of the I-BFM Study Group. Br J Haematol. 2024;205(1):268–79. https://doi.org/10.1111/bjh.19535

23. Berger M, Lanino E, Cesaro S, Zecca M, Vassallo E, Faraci M, De Bortoli M, Barat V, Prete A, Fagioli F. Feasibility and outcome of haploidentical hematopoietic stem cell transplantation with post-transplant high-dose cyclophosphamide for children and adolescents with hematologic malignancies: an AIEOP-GITMO Retrospective Multicenter Study. Biol Blood Marrow Transplant. 2016;22(5):902–9. https://doi.org/10.1016/j.bbmt.2016.02.002

24. Millot F, Ampatzidou M, Moulik NR, Tewari S, Elhaddad A, Hammad M, Pichler H, Lion T, Tragiannidis A, Shima H, An W, Yang W, Karow A, Farah R, Luesink M, Dworzak M, Sembill S, De Moerloose B, Sedlacek P, Schultz KR, Kalwak K, Versluys B, Athale U, Hijiya N, Metzler M, Suttorp M. Management of children and adolescents with chronic myeloid leukemia in chronic phase: International pediatric chronic myeloid leukemia expert panel recommendations. Leukemia. 2025;39(4):779–91. https://doi.org/10.1038/s41375-025-02543-4

25. Millot F, Suttorp M, Ragot S, Leverger G, Dalle JH, Thomas C, Cheikh N, Nelken B, Poirée M, Plat G, Versluys B, Lausen B, Borisevich M. Discontinuation of imatinib in children with chronic myeloid leukemia: a study from the International Registry of Childhood CML. Cancers (Basel). 2021;13(16):4102. https://doi.org/10.3390/cancers13164102

26. Chalandon Y, Saruerda A, Corbacioglu S, Greco R, Kroger N, Carreras E. Chronic myeloid leukemia. In: The EBMT Handbook. Europe: EBMT; 2024. p. 771–6.

27. Schmidt S, Liu Y, Hu ZH, Williams KM, Lazarus HM, Vij R, Kharfan-Dabaja MA, Ortí G, Wiernik PH, Weisdorf D, Kamble RT, Herzig R, Wirk B, Cerny J, Bacher U, Chaudhri NA, Nathan S, Farhadfar N, Aljurf M, Gergis U, Szer J, Seo S, Hsu JW, Olsson RF, Maharaj D, George B, Hildebrandt GC, Agrawal V, Nishihori T, Abdel-Azim H, Alyea E, Popat U, Sobecks R, Scott BL, Holter Chakrabarty J, Saber W. The role of donor lymphocyte infusion (DLI) in post-hematopoietic cell transplant (HCT) relapse for chronic myeloid leukemia (CML) in the tyrosine kinase inhibitor (TKI) era. Biol Blood Marrow Transplant. 2020;26(6):1137–43. https://doi.org/10.1016/j.bbmt.2020.02.006

28. National Comprehensive Cancer Network. NCCN Clinical Practice Guidelines in Oncology: chronic myeloid leukemia. Version 3. NCCN; 2025.