

# PEDIATRIC HEMATOLOGY: TYPES OF LEUKEMIA AND MODERN TREATMENT APPROACHES

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## Abstract

This article examines the major types of leukemia observed in pediatric patients and highlights modern treatment approaches currently utilized in clinical practice. It provides an overview of acute and chronic forms of childhood leukemia, including Acute Lymphoblastic Leukemia (ALL), Acute Myeloid Leukemia (AML), Chronic Myeloid Leukemia (CML), and rare subtypes. Special attention is given to recent advancements in diagnostics, targeted therapy, immunotherapy, and bone marrow transplantation. The article aims to present updated, evidence-based information to assist healthcare professionals in understanding current trends in pediatric leukemia management.

**Keywords:** Pediatric hematology; childhood leukemia; Acute Lymphoblastic Leukemia (ALL); Acute Myeloid Leukemia (AML); Chronic Myeloid Leukemia (CML); immunotherapy; targeted therapy; bone marrow transplantation; modern treatment.

## Introduction

Pediatric hematology is a rapidly developing field that focuses on diagnosing and treating blood-related disorders in children. Among these conditions, leukemia remains the most common childhood malignancy, accounting for a significant proportion of pediatric cancer cases worldwide. Leukemia in children presents with diverse clinical features depending on its type, severity, and progression rate. Early diagnosis and timely intervention are considered essential to improving survival outcomes. Recent decades have introduced major breakthroughs in the treatment of pediatric leukemia. Advanced laboratory diagnostics, genetic profiling, targeted medications, and cellular immunotherapies have significantly increased remission rates and overall survival. Despite these advancements, leukemia continues to pose clinical challenges due to its heterogeneous nature and potential treatment resistance. Therefore, understanding the classification of leukemia and the principles of modern treatment approaches is crucial for healthcare providers and researchers working in pediatric hematology.

Acute Lymphoblastic Leukemia (ALL) is the most common pediatric malignancy and accounts for the majority of childhood leukemia cases. It is characterized by the uncontrolled proliferation of lymphoblasts and is classified into B-cell and T-cell subtypes. B-cell ALL is more frequently diagnosed in younger children, while T-cell ALL is more typical in older pediatric patients. Clinically, the disease manifests with fatigue, bone pain, lymphadenopathy, and increased susceptibility to infections. Acute Myeloid Leukemia (AML) is less common in children but is known for its aggressive behavior. AML develops due to the malignant proliferation of myeloid



precursor cells. The disease often presents with excessive bleeding, anemia, recurrent infections, and bone marrow failure. Certain genetic abnormalities, such as  $t(8;21)$  and  $inv(16)$ , are associated with a more favorable prognosis. Morphological analysis, flow cytometry, and molecular testing are essential diagnostic tools for confirming AML. Chronic Myeloid Leukemia (CML) is rare in the pediatric population but may progress from a chronic phase to a blast crisis. The hallmark of CML is the presence of the Philadelphia chromosome, resulting from the BCR–ABL1 gene fusion. In children, CML typically develops gradually and presents with fatigue, marked leukocytosis, and splenomegaly. Molecular analysis is the primary method for confirming the diagnosis.

Chemotherapy remains the cornerstone of pediatric leukemia treatment. In ALL, treatment consists of induction, consolidation, and maintenance phases, each designed to eliminate malignant cells at different stages. In AML, however, treatment relies on intensive induction chemotherapy followed by multiple cycles of consolidation. Advances in chemotherapy protocols have significantly increased remission rates and improved overall outcomes in children. Targeted therapy focuses on specific genetic mutations within leukemic cells and has become a major component of modern treatment. For example, tyrosine kinase inhibitors — including imatinib, dasatinib, and nilotinib — effectively suppress the activity of the BCR–ABL1 protein in CML. In both ALL and AML, additional targeted agents are being used to inhibit disease-specific genetic alterations. Compared to traditional chemotherapy, targeted therapy is often more precise and better tolerated. Immunotherapy has emerged as one of the most innovative breakthroughs in pediatric leukemia treatment. One of the most significant advances is CAR-T cell therapy, particularly effective for relapsed or refractory B-cell ALL. Monoclonal antibodies, such as rituximab and blinatumomab, also enhance the immune system's ability to recognize and destroy leukemic cells by targeting specific surface antigens. Bone Marrow Transplantation (BMT) is recommended for high-risk cases of ALL and AML, as well as for CML patients who do not respond adequately to targeted therapy. During BMT, healthy hematopoietic stem cells from a donor are transplanted into the patient to restore normal blood formation. Although effective, this approach carries potential complications, including graft-versus-host disease (GVHD), which requires careful long-term monitoring.

Flow cytometry is essential for identifying the immunophenotype of malignant cells, helping clinicians differentiate leukemia subtypes and select appropriate treatment strategies.

Molecular diagnostic methods detect genetic markers such as BCR–ABL1, FLT3, and NPM1. These markers play an important role in determining risk classification and selecting personalized treatment options.

Minimal Residual Disease (MRD) monitoring allows clinicians to detect extremely small numbers of leukemic cells that may remain after treatment. MRD levels are crucial for assessing the risk of relapse and guiding further therapy decisions.

Genomic profiling provides a comprehensive analysis of mutations present in leukemic cells. This advanced technique supports precision medicine by helping clinicians choose the most effective individualized treatment approach.

Risk stratification plays a crucial role in determining treatment strategies for pediatric leukemia patients. Risk groups are assessed based on the child's age, laboratory findings, genetic abnormalities, and the initial response to therapy. For instance, in ALL, certain chromosomal changes such as  $t(12;21)$  and hyperdiploidy are associated with a favorable prognosis, while



abnormalities like BCR–ABL1 and MLL rearrangements indicate high-risk disease. Accurate risk assessment allows clinicians to tailor treatment intensity to the child’s individual needs and avoid unnecessary toxicity.

Throughout leukemia treatment, children may experience several adverse effects. Chemotherapy commonly causes immune suppression, anemia, thrombocytopenia, oral mucositis, and gastrointestinal disturbances. Therefore, supportive care — including infection prevention, transfusion of blood components, pain management, and proper nutritional support — is an essential part of therapy. Patients undergoing immunotherapy or CAR-T cell therapy may develop cytokine release syndrome, neurological complications, and other immune-related reactions. To manage these conditions, specific clinical protocols involving corticosteroids and immunomodulatory agents are used. Early detection and timely management of side effects significantly improve treatment outcomes.

Even after achieving complete remission, long-term follow-up is necessary for pediatric leukemia survivors. Monitoring bone marrow function, immune recovery, and physical growth is essential. Some children may experience late effects of chemotherapy, such as growth delay, thyroid dysfunction, or cardiotoxicity. Therefore, coordinated care involving pediatric hematologists, endocrinologists, cardiologists, and psychologists is recommended. Psychological rehabilitation also plays an important role. After prolonged treatment, children may face anxiety, fear, concentration problems, or memory difficulties. Rehabilitation programs are beneficial in restoring emotional balance and supporting the child’s social reintegration.

Survival rates for pediatric leukemia have increased significantly. In ALL, modern treatment protocols have led to overall survival rates reaching 85–90%. Although outcomes in AML remain lower, advances in drug therapy and bone marrow transplantation have improved survival to approximately 60–70%. In CML, the introduction of tyrosine kinase inhibitors has transformed the prognosis, providing effective long-term disease control. Despite these achievements, relapse remains a challenge in high-risk groups. Therefore, molecular diagnostics, MRD monitoring, and personalized therapy remain central components of global pediatric leukemia management strategies.

### **Conclusion.**

Childhood leukemia remains one of the most significant hematological disorders requiring timely diagnosis and comprehensive treatment. Advances in modern medical science — including chemotherapy, targeted therapy, immunotherapy, and bone marrow transplantation — have greatly improved survival outcomes and enhanced the quality of life for pediatric patients. Each type of leukemia presents distinct biological and clinical characteristics; therefore, accurate classification through advanced diagnostic techniques such as flow cytometry, molecular testing, and minimal residual disease monitoring is essential for selecting the most effective therapeutic strategy. The integration of precision medicine, supported by genomic profiling, has opened new opportunities for individualized treatment approaches, ensuring that therapies are tailored to the specific genetic and molecular features of each patient. Continued research and the development of innovative therapeutic modalities remain crucial for further improving prognosis, reducing treatment-related



complications, and achieving long-term remission. Overall, a multidisciplinary and personalized approach remains the cornerstone of successful management in pediatric leukemia.

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