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Perspective

Targeted Therapies in Acute Myeloid Leukemia: Mechanisms and Clinical Application

Jake Williams*

Department of Oncology, Mount Kenya University, Thika, Kenya

DESCRIPTION

Acute leukemia is a swift and aggressive form of blood cancer that often strikes without warning and progresses at a pace that demands immediate medical attention. Unlike its chronic counterparts that may evolve over months or even years, acute leukemia is relentless. It disrupts the body's normal function in a matter of days or weeks, leaving patients and their families in a whirlwind of diagnoses, treatment decisions, and emotional upheaval. This form of leukemia does not merely affect blood it upends entire lives.

At its core, acute leukemia begins in the bone marrow, the soft tissue inside bones responsible for producing blood cells. In healthy individuals, this process is tightly regulated, producing just the right balance of red cells to carry oxygen, white cells to fight infection, and platelets to prevent bleeding. In acute leukemia, this balance is shattered. Immature white blood cells blasts begin to multiply uncontrollably. These non-functioning cells flood the bone marrow and bloodstream, crowding out the healthy cells and disrupting normal hematologic processes. The body is left vulnerable, struggling to carry oxygen, clot wounds, and ward off infections.

There are two major types of acute leukemia: Acute Lymphoblastic Leukemia (ALL) and Acute Myeloid Leukemia (AML). While they differ in the specific cell lines they affect ALL targets lymphoid cells, and AML affects myeloid cells they share common traits of rapid progression and an urgent need for treatment. ALL is more common in children, and with recent advances, survival rates have improved dramatically for pediatric cases. AML, more prevalent in adults, remains a challenging disease, particularly in older patients with co-existing health issues. The urgency with which both types demand intervention is daunting; delays of even a few days can allow the disease to advance significantly.

For those diagnosed, the experience is often a jarring rupture in the rhythm of life. One day, an individual may feel a bit more tired than usual or notice unusual bruising or frequent nosebleeds. A blood test is ordered, often to rule out something minor. And then the bottom falls out. A call from a doctor requesting an immediate hospital admission, a sudden flood of terminology bone marrow biopsy, blast count, chemotherapy protocol quickly replaces everyday worries with life-altering ones. Plans are canceled. Jobs are put on hold. The future becomes uncertain.

From the patient's perspective, the battle against acute leukemia is fought on several fronts. There is the physical toll of the disease itself profound fatigue, vulnerability to infections, spontaneous bleeding and the side effects of treatments which are often aggressive and debilitating. Induction chemotherapy, the first phase of treatment, aims to rapidly eliminate leukemic cells. This process usually requires weeks of hospitalization and causes severe immune suppression. Patients often endure mouth sores, nausea, fevers, and hair loss while confined to isolation wards. They are monitored continuously for infections that could prove fatal due to a lack of immune defense.

Beyond the hospital walls, acute leukemia places a tremendous emotional and psychological burden on patients and families. The pace at which decisions must be made whether to proceed with chemotherapy, consider a bone marrow transplant, or enter a clinical trial leaves little time to process the diagnosis. Many grapple with anxiety, depression, and feelings of helplessness. Families become caregivers overnight, managing medications, transportation to treatment centers, financial burdens, and emotional support. The disease tests the strength of relationships and the resilience of even the most stoic individuals.

In adult populations, however, the fight remains more complex. AML, in particular, has a dismal prognosis for older patients, with five-year survival rates hovering around 25%. The heterogeneity of the disease driven by a wide array of genetic mutations and chromosomal abnormalities makes it difficult to treat with a uniform approach. Recent advances in genomic sequencing have shed light on the molecular underpinnings of AML, leading to the development of targeted therapies such as FLT3 and IDH inhibitors. These drugs offer hope for patients

Correspondence to: Jake Williams, Department of Oncology, Mount Kenya University, Thika, Kenya, E-mail: williamsj@gmail.com

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whose cancers are driven by specific mutations, providing alternatives to traditional chemotherapy. Still, the challenge lies in tailoring treatments effectively while managing side effects and ensuring access to these novel options.