

# acute myeloid leukemia targeted therapy

Acute Myeloid Leukemia Targeted Therapy: Revolutionizing Treatment and Outcomes

**acute myeloid leukemia targeted therapy** has emerged as a groundbreaking approach in the fight against this aggressive blood cancer. Unlike traditional chemotherapy, which broadly attacks rapidly dividing cells, targeted therapy zeroes in on specific genetic mutations and molecular pathways driving the growth of leukemia cells. This precision medicine strategy is transforming treatment paradigms, offering new hope for improved survival and quality of life for patients diagnosed with acute myeloid leukemia (AML).

Understanding the landscape of AML and the role of targeted therapies requires exploring the biology of the disease, the molecular targets currently being exploited, and the latest advancements shaping patient care. In this article, we'll delve deep into how acute myeloid leukemia targeted therapy works, the different types of agents available, and what the future holds for this promising field.

## What Is Acute Myeloid Leukemia and Why Targeted Therapy Matters

Acute myeloid leukemia is a fast-progressing cancer of the bone marrow and blood characterized by the uncontrolled proliferation of abnormal myeloid cells. These immature cells crowd out healthy blood cells, leading to symptoms such as fatigue, infections, anemia, and bleeding. AML is known for its genetic complexity, with many patients harboring distinct mutations that influence disease behavior and treatment response.

Traditional AML treatment often involves intensive chemotherapy regimens aimed at killing rapidly dividing cells. While chemotherapy can induce remission, it is not selective and can cause significant side effects due to collateral damage to normal cells. Additionally, many patients relapse or become resistant to these treatments.

This is where acute myeloid leukemia targeted therapy comes into play. By employing drugs that specifically inhibit molecular abnormalities driving leukemia growth, targeted therapies offer a more personalized and often less toxic alternative. This approach has opened doors to therapies that not only improve remission rates but also extend survival in subsets of AML patients.

## Key Molecular Targets in AML for Targeted Therapy

To appreciate how targeted therapy works, it helps to understand the specific molecules and pathways involved in AML pathogenesis. Several genetic mutations have been identified as critical drivers of AML, and many of these serve as therapeutic targets.

## **FLT3 Mutations and FLT3 Inhibitors**

One of the most common mutations in AML occurs in the FLT3 gene, which encodes a receptor tyrosine kinase involved in cell growth. FLT3 mutations, particularly internal tandem duplications (ITD), are associated with poor prognosis. Targeted drugs called FLT3 inhibitors block the aberrant signaling caused by these mutations.

Examples include midostaurin and gilteritinib, which have shown efficacy in improving outcomes when combined with chemotherapy or used as monotherapy in relapsed AML. By selectively inhibiting FLT3, these agents reduce leukemia proliferation without affecting healthy cells as broadly as chemotherapy.

## **IDH1 and IDH2 Mutations**

Mutations in the IDH1 and IDH2 genes alter cellular metabolism, leading to the production of an oncometabolite that promotes leukemia cell survival. Targeted inhibitors such as ivosidenib (IDH1 inhibitor) and enasidenib (IDH2 inhibitor) have been developed to reverse this abnormality.

These drugs have been approved for relapsed or refractory AML patients harboring the respective mutations and have demonstrated durable responses with relatively manageable side effects.

## **BCL-2 Protein and Apoptosis Modulation**

The BCL-2 protein plays a crucial role in preventing programmed cell death (apoptosis), enabling cancer cells to survive longer than they should. Venetoclax, a BCL-2 inhibitor, has made significant strides in AML treatment, particularly when combined with hypomethylating agents or low-dose chemotherapy for older patients or those unfit for intensive regimens.

By promoting apoptosis in leukemic cells, venetoclax-based regimens have improved remission rates and extended survival in difficult-to-treat AML populations.

## **Emerging and Novel Therapies in AML Targeted Treatment**

The field of acute myeloid leukemia targeted therapy continues to evolve rapidly, with numerous investigational agents and approaches under clinical evaluation.

## **Menin Inhibitors**

Menin is a protein involved in regulating gene expression, and recent research has identified its role in leukemias driven by MLL gene rearrangements and NPM1 mutations. Menin inhibitors aim to disrupt this interaction, potentially halting leukemia progression.

Early-phase trials have shown promise, and these agents may become part of future targeted regimens.

## **Antibody-Drug Conjugates (ADCs)**

ADCs combine the specificity of monoclonal antibodies with potent chemotherapy agents, delivering cytotoxic drugs directly to leukemia cells expressing certain surface markers. For example, gemtuzumab ozogamicin targets CD33, a protein commonly found on AML cells.

This targeted delivery reduces systemic toxicity and enhances treatment effectiveness.

## **Immunotherapies and Checkpoint Inhibitors**

Harnessing the immune system to fight AML is another exciting frontier. Checkpoint inhibitors that block molecules suppressing immune responses are being tested alone or combined with other therapies to boost anti-leukemia immunity.

While still in early stages for AML, these immunotherapeutic strategies hold potential to complement targeted therapy.

## **Integrating Targeted Therapy into AML Treatment Plans**

Deciding on the right treatment approach in AML involves a thorough assessment of the patient's age, overall health, genetic mutations, and disease characteristics. Molecular testing is now standard to identify actionable mutations that qualify patients for targeted therapies.

In many cases, targeted agents are combined with chemotherapy or hypomethylating agents to enhance effectiveness. For example, midostaurin is added to induction chemotherapy in FLT3-mutated AML, while venetoclax is paired with azacitidine in older patients.

This integration requires careful monitoring for side effects and response, as well as adjustments based on minimal residual disease status and relapse risk.

## Benefits and Considerations

Targeted therapies generally offer:

- Increased specificity toward leukemia cells
- Reduced toxicity compared to traditional chemotherapy
- Improved response rates in genetically defined AML subsets
- Potential for oral administration and outpatient management

However, challenges include the development of resistance, side effects unique to targeted agents, and the need for comprehensive genetic profiling.

## The Future of Acute Myeloid Leukemia Targeted Therapy

The horizon for AML treatment looks increasingly personalized. Advances in genomic technologies continue to uncover new mutations and biological pathways that can be exploited therapeutically. Combination strategies pairing multiple targeted agents or integrating immunotherapies are under active investigation.

Moreover, the use of minimal residual disease monitoring and real-time molecular profiling will help tailor therapy more precisely, improving outcomes and minimizing unnecessary toxicity.

Researchers are also working toward identifying biomarkers predictive of response and resistance, which will refine patient selection and treatment sequencing.

Acute myeloid leukemia targeted therapy has already changed the landscape for many patients, turning what was once a uniform and often bleak prognosis into a more hopeful scenario with individualized options. As science progresses, the promise of turning AML into a manageable or even curable disease grows stronger, underscoring the importance of ongoing research and patient access to molecular testing and targeted treatments.

## Frequently Asked Questions

### What is targeted therapy in the treatment of acute myeloid leukemia (AML)?

Targeted therapy in AML refers to treatments that specifically target genetic mutations or

proteins involved in the growth and survival of leukemia cells, aiming to minimize damage to normal cells.

## **Which genetic mutations in AML are commonly targeted by current therapies?**

Commonly targeted mutations in AML include FLT3, IDH1, and IDH2 mutations, each of which has specific inhibitors approved or in clinical trials.

## **What are FLT3 inhibitors and how do they work in AML treatment?**

FLT3 inhibitors, such as midostaurin and gilteritinib, block the FLT3 protein tyrosine kinase activity, which is often mutated and overactive in some AML patients, thereby reducing leukemia cell proliferation.

## **Are IDH1 and IDH2 inhibitors effective in AML targeted therapy?**

Yes, IDH1 inhibitors like ivosidenib and IDH2 inhibitors like enasidenib have shown efficacy in targeting mutated IDH enzymes in AML, leading to differentiation and death of leukemia cells.

## **How does targeted therapy compare to traditional chemotherapy in AML?**

Targeted therapy tends to have fewer side effects and can be more effective in patients with specific mutations, whereas traditional chemotherapy is less specific and often more toxic but remains a standard treatment for many AML patients.

## **Can targeted therapy be combined with other treatments in AML?**

Yes, targeted therapies are often combined with chemotherapy, hypomethylating agents, or other targeted drugs to improve treatment outcomes in AML patients.

## **What are some emerging targeted therapies currently in clinical trials for AML?**

Emerging therapies include menin inhibitors, venetoclax combinations, and novel agents targeting other mutations or pathways such as TP53 or BCL-2 in AML.

## **What role does molecular testing play in selecting targeted therapies for AML?**

Molecular testing identifies specific genetic mutations in AML cells, guiding clinicians to

select appropriate targeted therapies tailored to the patient's leukemia profile.

## **Are there any limitations or challenges associated with targeted therapy in AML?**

Challenges include the development of resistance to targeted agents, limited efficacy in AML cases without targetable mutations, and the high cost of some therapies.

## **Additional Resources**

Acute Myeloid Leukemia Targeted Therapy: Advancements and Clinical Perspectives

**acute myeloid leukemia targeted therapy** represents a significant evolution in the treatment paradigm for a complex and aggressive hematologic malignancy. Unlike conventional chemotherapy, which often indiscriminately affects both cancerous and healthy cells, targeted therapies aim to interfere with specific molecular pathways driving leukemic cell proliferation and survival. This precision medicine approach has transformed the landscape of acute myeloid leukemia (AML) management, offering new hope for improved outcomes in a disease historically associated with poor prognosis.

## **Understanding Acute Myeloid Leukemia and the Rationale for Targeted Therapy**

AML is characterized by the rapid clonal expansion of myeloid precursors in the bone marrow, leading to impaired hematopoiesis and subsequent cytopenias. The heterogeneity of AML at the genetic and molecular level has been well documented, with numerous mutations and chromosomal abnormalities contributing to disease pathogenesis.

Traditional chemotherapy regimens, such as the “7+3” protocol combining cytarabine and an anthracycline, remain the backbone of treatment for many patients but are often limited by toxicity and resistance.

The advent of molecular profiling technologies has enabled the identification of actionable mutations and aberrant signaling pathways in AML cells. This has paved the way for acute myeloid leukemia targeted therapy, focusing on inhibiting specific oncogenic drivers like FLT3, IDH1/IDH2, and BCL-2, among others. By exploiting these vulnerabilities, targeted agents aim to improve remission rates, reduce relapse, and minimize off-target effects.

## **Key Targets and Approved Agents in AML Targeted Therapy**

## **FLT3 Inhibitors**

FMS-like tyrosine kinase 3 (FLT3) mutations are among the most common genetic alterations in AML, present in approximately 30% of cases. These mutations, particularly internal tandem duplications (ITD), are associated with aggressive disease and poor prognosis. FLT3 inhibitors such as midostaurin and gilteritinib have been developed to block the aberrant kinase activity.

Midostaurin was the first FLT3 inhibitor approved for use in combination with standard chemotherapy in newly diagnosed FLT3-mutated AML, demonstrating a significant improvement in overall survival in the RATIFY trial. Gilteritinib, a second-generation FLT3 inhibitor with greater specificity, is approved for relapsed or refractory FLT3-mutated AML, showing enhanced efficacy and a more favorable side effect profile compared to earlier agents.

## **IDH1 and IDH2 Inhibitors**

Mutations in isocitrate dehydrogenase enzymes IDH1 and IDH2 occur in roughly 20% of AML patients and result in the production of the oncometabolite 2-hydroxyglutarate, which impairs normal cell differentiation. Targeted inhibitors such as ivosidenib (IDH1) and enasidenib (IDH2) have demonstrated the ability to induce differentiation and remission in patients with relapsed or refractory AML harboring these mutations.

Clinical trials have revealed that these agents not only offer a new therapeutic option but also tend to have a more tolerable safety profile compared to traditional chemotherapy. However, differentiation syndrome remains a notable adverse event requiring prompt recognition and management.

## **BCL-2 Inhibitors**

The anti-apoptotic protein BCL-2 is frequently overexpressed in AML cells, contributing to chemoresistance and disease persistence. Venetoclax, a potent BCL-2 inhibitor, has emerged as a crucial drug in the targeted therapy arsenal, particularly when combined with hypomethylating agents or low-dose cytarabine in elderly or unfit patients.

This combination has shown remarkable response rates in treatment-naïve AML patients who are ineligible for intensive chemotherapy, significantly altering the therapeutic landscape and underscoring the clinical relevance of apoptosis modulation.

## **Comparative Efficacy and Challenges in Targeted AML Therapy**

While acute myeloid leukemia targeted therapy has introduced promising treatment

avenues, it is essential to contextualize these advances within the broader clinical framework. Targeted agents often yield higher specificity and reduced systemic toxicity but may be limited by the development of resistance mechanisms and variable patient responses.

For example, FLT3 inhibitors, despite initial success, can encounter secondary mutations that diminish drug efficacy. Similarly, IDH inhibitors can lead to incomplete remissions or relapse, necessitating combination strategies or sequential therapies. Venetoclax-based regimens, although effective, require careful monitoring of tumor lysis syndrome and cytopenias.

Furthermore, the cost and accessibility of these novel agents pose practical challenges, particularly in resource-limited settings. Integrating targeted therapies with existing treatment protocols demands a multidisciplinary approach, incorporating molecular diagnostics, risk stratification, and patient-specific considerations.

## **Emerging Targets and Future Directions**

Ongoing research continues to expand the repertoire of molecular targets in AML. Agents targeting menin inhibitors, which disrupt epigenetic regulation in MLL-rearranged AML, and inhibitors of checkpoint kinases are in various stages of clinical development. Moreover, the exploration of immunotherapeutic modalities, including bispecific antibodies and CAR-T cells directed at AML antigens, represents a complementary frontier.

Personalized medicine, driven by comprehensive genomic profiling, is poised to refine patient selection and optimize therapeutic combinations. The integration of minimal residual disease monitoring through molecular techniques further assists in tailoring treatment duration and intensity.

## **Clinical Implementation and Patient Considerations**

Implementing acute myeloid leukemia targeted therapy requires a nuanced understanding of molecular diagnostics and patient-specific factors such as age, performance status, and comorbidities. Molecular testing for FLT3, IDH1/2, and other relevant mutations should ideally be performed at diagnosis to guide treatment decisions.

Physicians must also balance the benefits of targeted agents with potential toxicities and the risk of resistance. Patient education regarding adherence, side effect recognition, and supportive care is critical. Multidisciplinary teams including hematologists, pharmacists, and nursing staff play a pivotal role in managing therapy and improving quality of life.



## Integration with Conventional Therapies

Targeted therapies have not supplanted chemotherapy or hematopoietic stem cell transplantation but rather complement these modalities. For instance, midostaurin is administered alongside induction chemotherapy, while venetoclax combinations offer less intensive alternatives. In patients eligible for transplantation, targeted therapy can serve as a bridge to transplant or maintenance post-transplant to reduce relapse risk.

## Conclusion: The Evolving Landscape of AML Treatment

Acute myeloid leukemia targeted therapy signifies a paradigm shift from broad-spectrum cytotoxic drugs to precision medicine tailored to individual molecular profiles. While challenges remain—including resistance, side effects, and accessibility—the integration of targeted agents into clinical practice has improved remission rates and survival outcomes.

The continued evolution of molecular diagnostics and the development of novel therapeutic targets promise to further refine AML management. As research advances, a more personalized, effective, and tolerable approach to treating acute myeloid leukemia appears increasingly attainable, offering renewed optimism for patients and clinicians alike.

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Michael Andreeff, 2014-11-20 This book provides an unprecedented overview of Targeted Therapies for acute myeloid leukemias. It aims at an almost comprehensive coverage of the diverse therapeutic strategies that have been developed during the last decade and are now being evaluated in early clinical trials. Paired and authoritative chapters by leading research scientists and clinicians explain basic concepts and clinical translation of topics that include the underlying genetic and proteomic abnormalities of AML, the development of novel nucleoside analogues, the roles of microRNAs, apoptosis regulators Bcl-2 and p53 and of critical cell signaling proteins such as PIM, FLT3, Raf/MEK, PI3K/AKT/mTOR and aurora kinases. Chapters on epigenetic mechanisms, nuclear receptors, cell surface antigens, the hypoxic leukemia microenvironment, stem cells and leukemia metabolism provide insights into leukemia cell vulnerabilities. Cell therapies utilizing T-, NK- and mesenchymal stem cells and progress in hematopoietic transplantation strategies round up this overview of the multi-dimensional therapeutic landscape in which leukemia specialists develop treatment strategies that are expected to make leukemia history in the near future.

**acute myeloid leukemia targeted therapy:** Targeted Therapy in Translational Cancer Research Apostolia-Maria Tsimberidou, Razelle Kurzrock, Kenneth C. Anderson, 2015-12-21

Targeted Therapy in Translational Cancer Research for the Translational Oncology series provides a comprehensive overview of recent developments in our understanding of tumor biology, elucidates the roles of targets and pathways involved in carcinogenesis, and describes current state-of-the-art anticancer therapy, as well as the most promising areas of translational research and targeted therapy. Introduces cutting-edge 'bench to bedside and back' breakthroughs which have transformed the diagnosis, prognosis, and treatment of cancer Covers basic principles of targeted therapy, including immunotherapy and the roles of cancer stem cells, the microenvironment, angiogenesis, epigenetics, microRNAs, and functional imaging in precision medicine Summarises major advances in therapeutic management of hematologic malignancies and solid tumors using conventional therapy, targeted therapy, immunotherapy, or novel treatment modalities

**acute myeloid leukemia targeted therapy:** Molecularly Targeted Therapy for Childhood Cancer Peter J. Houghton, Robert J. Arceci, 2010-09-10 Each chapter will focus on the known molecular characteristics of specific childhood cancers, focusing on how the molecular 'drivers' can be exploited from a therapeutic standpoint with currently available targeted agents. Where applicable, integration of targeted therapies with conventional cytotoxic agents will be considered. This volume will provide a comprehensive summary of molecular characteristics of childhood cancers, and how the changes involved in transformation provide us with opportunities for developing relatively less toxic, but curative, therapies.

**acute myeloid leukemia targeted therapy:** Cancer Stem Cell Resistance to Targeted Therapy Cristina Maccalli, Matilde Todaro, Soldano Ferrone, 2019-06-04 This book represents an up-dated summary of the state of the art of the characterization of cancer stem cell/ cancer initiating cell (CSC/CIC) properties. An overview of the definition and biological properties of CSCs/CICs as well as the role of these cells in determining the resistance to standard and immune-based therapies is provided. It also discusses limitations in the achievement of a definitive biological characterization of CSCs/CICs due to their high extent of plasticity and heterogeneity that is also mutually driven by the interaction of these cells with the tumor microenvironment. The limitations in targeting CSCs/CICs with immunotherapy are also explained together with explorative combination approaches that could increase the susceptibility of these cells to the recognition by immune cells. This book is conceived for a broad audience, including students, teachers, scientific experts. The critical revision of available results in terms of immunological profile of CSCs/CICs and the efficacy in targeting these cells by immunological approaches, results in a comprehensive and up to date recapitulation of the field and provides interesting suggestions on how to focus future investigations in order to assess the role of CSCs/CICs as prognostic and predictive biomarkers of responsiveness to therapies for cancer patients.

**acute myeloid leukemia targeted therapy:** Targeted Therapies in Cancer Manfred Dietel, 2007-06-10 From its introduction, oncological chemotherapy has been encumbered by its poor selectivity because most antiproliferative drugs are toxic not only to tumor cells but also to important populations of the body's non-neoplastic cells. The resultant problems with unwanted side effects are compounded by difficulties in predicting the desired effectivity of chemotherapy in individual patients. This unsatisfactory situation has driven intensive research and development towards more specific and less toxic anticancer drugs over the last few decades. Several results of these efforts have reached the clinic and an even greater number are now in preclinical testing. Common to all these targeted therapies is their interaction with defined molecules present on cancer cells, which adds various degrees of increased selectivity to their toxic effects. As a consequence, detecting the target molecule on tumors before therapy holds great diagnostic potential for predicting the efficacy of the drug and personalizing therapy. This book aims to present translational scientists and clinicians with an integrated critical view on the theories, mechanisms, problems and pitfalls of the targeted therapy approach.

**acute myeloid leukemia targeted therapy:** Acute Leukemias Pier Paolo Piccaluga,

2021-03-24 Written by expert research teams, this book describes different aspects of both acute myeloid and acute lymphocytic leukemia, specifically their pathobiology, classification/diagnosis, and treatment. Chapters highlight current research as well as the gold standards for diagnosis and treatment of these diseases, examining recent advances in personalized approaches to acute leukemia.

**acute myeloid leukemia targeted therapy: 365 Days of progress in cancer molecular targets and therapeutics** Wafik S. El-Deiry, Massimo Broggin, 2023-09-26

**acute myeloid leukemia targeted therapy: Resistance of Targeted Therapies Excluding Antibodies for Lymphomas** Andrés J. M. Ferreri, 2018-04-04 In the last decade, the literature on molecular mechanisms and activated pathways in the different lymphoma categories increased exponentially, which was followed by a more diffuse and successful use of targeted therapies. In this book, expert authors revisit the most relevant aspects of these therapies, with special emphasis on molecular mechanisms and clinical effects of resistance. The knowledge of the underlying mechanisms involved in tumor resistance to target therapies is of paramount importance because they will result in a better selection of patients with sensitive disease and the establishment of suitable combinations of drugs that target different molecules and could overcome the established resistance.

**acute myeloid leukemia targeted therapy: Special Issue on Innovative Multi-Disciplinary Approaches for Precision Studies in Leukemia** Xu Huang, Sandra Marmiroli, Alison M. Michie, Valentina Serafin, 2021-10-01

**acute myeloid leukemia targeted therapy: Manual of Hematopoietic Cell Transplantation and Cellular Therapies - E-Book** Qaiser Bashir, Elizabeth J. Shpall, Richard E. Champlin, 2022-12-23 Led by authors from MD Anderson's Stem Cell Transplantation and Cellular Therapy Department, the world's largest and highly respected program at the forefront of rapidly advancing treatments in the field, Manual of Hematopoietic Cell Transplantation and Cellular Therapies is a comprehensive, focused reference covering the latest clinical developments and applications of stem cell transplant and cellular therapies for hematologic malignancies and solid tumors. This cutting-edge title, with a majority contribution from the MD Anderson Cancer Center and leading faculty from other academic institutions, covers breakthrough cell-based therapies for various diseases including lymphoma, multiple myeloma, leukemia, and select solid tumor and autoimmune diseases. This unique, definitive resource is essential for hematologists, fellows in hematology and immunotherapy, mid-level providers, pharmacists, and oncologists who refer patients for cell-based therapies. - Addresses hematologic conditions including leukemia, lymphoma, and myeloma. - Offers guidance on hematopoietic cell transplantation for solid tumors. - Covers basic science principles, clinical aspects, pharmacology, radiation therapy, and disease-specific guidelines, including prevention and management of complications. - Discusses key topics such as hematopoietic cell collection, bone marrow harvesting, umbilical cord blood transplantation, CAR T-cell therapy, and patient/donor selection and preparation of HCT. - Features extensive summary boxes, bulleted content, and algorithms throughout for quick and easy reference. - Offers team-based, clinically-focused coverage from world-renowned leaders in the field.

**acute myeloid leukemia targeted therapy: Brody's Human Pharmacology - E-Book** Lynn Wecker, Susan L. Ingram, 2024-02-02 Brody's Human Pharmacology is a well-established and much-loved textbook that guides students of medicine, pharmacology and other health professions through pharmacology from both basic and clinical perspectives. What can be a very difficult and complex topic is divided into easily digestible chapters, each of which is clearly structured from a therapeutic overview to mechanisms of action, relationships to clinical responses, pharmacokinetics, pharmacovigilance, new developments, and clinical relevance for all health professionals. - Contains all the essential aspects of pharmacology - suitable for exam preparation - Full color illustrations explain important processes and color-coded boxes enhance understanding - Therapeutic overviews, clinical problems and trade names - everything you need to know - Self-assessment questions and further reading to reinforce learning - Current developments for each class of drugs and how

information relates to students in the healthcare professions bring learning to life - Updated content throughout - Pharmacodynamics chapter revised to include discussions of positive and negative allosteric modulators, as well as biased agonism - Pharmacogenetics chapter considers genetic variations and their relationship to drug responses - Section on cancer drugs revised to reflect major advances - New chapter on immunological and cell-based therapies

**acute myeloid leukemia targeted therapy: Infectious Complications in Biologic and Targeted Therapies** Carlos Cervera, Jose Maria Aguado, 2022-11-18 This book aims to approach the epidemiology, diagnosis and management of infectious complications related to the use of biologic and targeted therapies. The first part is a general overview on the epidemiology and prevention of infections with the use of these agents. Timelines, pre-treatment evaluation, use of vaccines and travel counseling. In the second part, we will analyze the specific risk of infections associated with the use of the most important agents. The third part approach the problem on a syndromic point of view. For example, how to evaluate pulmonary infiltrates in a patient receiving biologic agents. This part III includes the most difficult and conflicting syndromes that clinicians may face when evaluating these patients. This book is intended not only for infectious diseases specialists, but also for different medical specialties prescribing biologic and targeted therapies.

**acute myeloid leukemia targeted therapy: Precision Pathology of Cancer** Xianfeng F. Zhao, Shiyong Li, 2024-11-05 Precision pathology of cancer is a new frontier - a systemic diagnostic approach to provide specific therapeutic targets and genetic and metabolic information for the treatment of patients. Not only are cancers extremely diverse but there is also heterogenetic variation among patients with the same cancer; owing to differences in genetic makeup and metabolic levels, a drug may work in one patient but be toxic in another. This text covers both basic concepts (the role of the precision pathologist, ethical issues, molecular basis, tools used in precision pathology, and specimen procurement) and clinical practice (the precise genes/pathways, ongoing clinical trials, therapeutics, pharmacogenomics, pharmacoproteomics, and pharmacometabolomics) in precision pathology of cancer. Covers both the conceptual bases and the clinical applications Enables pathologists, haematologists, and medical oncologists to apply Precision Pathology to their daily clinical practice Covers the whole range of cancers in practical detail

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**acute myeloid leukemia targeted therapy: Wintrobe's Clinical Hematology** John P. Greer, Daniel A. Arber, Bertil Glader, Alan F. List, Robert T. Means, Frixos Paraskevas, George M. Rodgers, 2013-08-29 With the 13th edition, Wintrobe's Clinical Hematology once again bridges the gap between the clinical practice of hematology and the basic foundations of science. Broken down into eight parts, this book provides readers with a comprehensive overview of: Laboratory Hematology, The Normal Hematologic System, Transfusion Medicine, Disorders of Red Cells, Hemostasis and Coagulation; Benign Disorders of Leukocytes, The Spleen and/or Immunoglobulins; Hematologic Malignancies, and Transplantation. Within these sections, there is a heavy focus on the

morphological exam of the peripheral blood smear, bone marrow, lymph nodes, and other tissues. With the knowledge about gene therapy and immunotherapy expanding, new, up-to-date information about the process and application of these therapies is included. Likewise, the editors have completely revised material on stem cell transplantation in regards to both malignant and benign disorders, graft versus host disease, and the importance of long-term follow-up of transplantation survivors.

**acute myeloid leukemia targeted therapy: Acute Myelogenous Leukemia** Lalitha Nagarajan, 2010-03-20 Acute myelogenous leukemia (AML), is the most common form of leukemia in adults. AML is a deadly form of malignancy, the prognosis for which has not improved in the last two decades. More importantly, it is a malignancy that is seen in older adults, therefore the number of cases is likely to rise as the population ages. Over the past 15 years, genetic mechanisms underlying AML have begun to unfold. Additional research in this area has helped identify key components and characteristics. Consequently, targeted therapy of AML is receiving much attention. It is the hope of researchers that as with chronic myelogenous leukemia (CML), and the drug, Gleevec, a targeted therapy for AML will be discovered.

**acute myeloid leukemia targeted therapy: Innovations in Development, Translational Research and Manufacturing of CAR T cells** Michael Hudecek, Qasim Rafiq, Stephen Goldrick, Ulrike Koehl, Helene Negre, Carmen Sanges, 2024-10-03 Immunotherapy with genetically engineered immune cell products is a transformative treatment modality with potential applications in various fields of medicine. A prime example is chimeric antigen receptor (CAR)-modified T cells in hematology and oncology, and the advent of CAR T cell therapies to treat infectious diseases, autoimmune disorders, and cardiovascular diseases. The medical need and demand from patients and caregivers require radical innovations to accelerate and improve pre-clinical development and clinical translation, provision of gene-transfer vectors, and immune cell product manufacturing as well as a critical reflection and discussion on ethical and socioeconomic aspects. The goal of this special issue of *Frontiers in Immunology* is to provide a comprehensive and multi-faceted view on the current state-of-the-art, imminent and future directions the field is taking in order to accelerate the pre-clinical development, clinical translation, and manufacturing of CAR T cells, increase access and sustainability of CAR T cell therapy for health care systems (in developed and in developing countries). This special issue will focus on the medical and scientific dimension incl. approved and emerging indications, new areas in medicine, advanced gene-transfer and gene-editing technologies, innovations in pre-clinical assessment (efficacy, toxicology, genomic safety), innovations in scalable automated manufacturing (bioprocessing), the implementation of high content data acquisition, machine learning and artificial intelligence, innovations in clinical trial design; and consider the ethical, socioeconomic and societal dimension of CAR T cells in particular and gene-engineered immune cell therapy in general.

**acute myeloid leukemia targeted therapy: Pathogenesis and Treatment of Leukemia** Harinder Gill, Yok-Lam Kwong, 2023-09-27 This book covers a comprehensive update on acute and chronic leukemia. In 54 chapters, authors introduce research progress and clinical trials of acute myeloid leukaemia (AML), acute promyelocytic leukaemia (APL), acute lymphoblastic leukaemia (ALL), myelodysplastic syndrome (MDS), myeloproliferative neoplasm (MPN) and chronic myeloid leukemia (CML). The last decade has seen the integration of genetic and clinical information to determine the prognosis and treatment strategies. This book provides practitioners, researchers and graduate students of Hematology and Hematopathology a comprehensive update on the pathobiology, genomics, classification, diagnosis, monitoring, prognostication and therapy of both acute and chronic leukemias.

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