Emerging therapeutic strategies for myelodysplastic syndromes: novel targets and approaches.

Charles Eliot*

Queen Elizabeth University, Glasgow Royal Infirmary, Scotland, UK

Abstract

Myelodysplastic syndromes (MDS) are a group of hematological disorders characterized by abnormal blood cell production in the bone marrow. While current treatment options for MDS include chemotherapy, immunomodulatory agents, and stem cell transplantation, there is a need for novel therapeutic approaches that target the underlying molecular mechanisms of the disease. In this mini review, we will discuss emerging therapeutic strategies for MDS, including epigenetic modifiers, immune checkpoint inhibitors, and targeted therapies, and their potential impact on patient outcomes.

Keywords: Myelodysplastic syndromes, Therapeutic strategies, Bone marrow.

Introduction

Myelodysplastic syndromes are a heterogeneous group of hematological disorders that arise from the clonal expansion of abnormal hematopoietic stem cells. The disease is characterized by ineffective hematopoiesis, resulting in low blood cell counts and a high risk of transformation to Acute Myeloid Leukemia (AML). While current treatment options for MDS include chemotherapy, immunomodulatory agents, and stem cell transplantation, these approaches are often limited by their toxicity and lack of efficacy in some patients [1]. Therefore, there is a need for novel therapeutic approaches that target the underlying molecular mechanisms of the disease.

Emerging therapeutic strategies

Epigenetic modifications are alterations to the DNA or chromatin structure that regulate gene expression without changing the DNA sequence [2]. Aberrant epigenetic changes have been implicated in the pathogenesis of MDS. Drugs that target these changes, such as DNA Methyltransferase Inhibitors (DNMTi) and Histone Deacetylase Inhibitors (HDACi), have shown promise in clinical trials. For example, azacitidine, a DNMTi, has been shown to improve overall survival in high-risk MDS patients and is now the standard of care in this population [3].

Immune checkpoint inhibitors

Immune checkpoint inhibitors are a class of drugs that block proteins on T cells that regulate immune responses. In MDS, immune checkpoint inhibitors have shown potential in enhancing the immune response against malignant cells [4]. For example, clinical trials have shown that pembrolizumab, a PD-1 inhibitor, can induce durable responses in a subset of MDS patients [5].

Targeted therapies

Targeted therapies are drugs that selectively target specific molecular pathways that are dysregulated in cancer cells. In MDS, several targeted therapies are currently under investigation, including inhibitors of the spliceosome machinery and IDH1/2 mutations. Spliceosome inhibitors, such as H3B-8800 and E7107, have shown promising results in preclinical studies and are currently in phase I clinical trials [6]. IDH1/2 inhibitors, such as ivosidenib and enasidenib, have been approved for the treatment of IDH1/2-mutated AML and are being investigated in MDS.

Conclusion

Emerging therapeutic strategies for MDS, including epigenetic modifiers, immune checkpoint inhibitors, and targeted therapies, offer new opportunities for the treatment of this complex disease. While some of these approaches have already shown promising results in clinical trials, further research is needed to fully understand their potential impact on patient outcomes. Moreover, the development of personalized treatment approaches that take into account individual patient characteristics, such as the presence of specific genetic mutations, will be critical for improving the efficacy of these novel therapies.

References

1. Makishima H, Yoshizato T, Yoshida K, et al. Dynamics of clonal evolution in myelodysplastic syndromes. Nat Genet. 2017;49:204–12.

***Correspondence to:** Charles Eliot, Queen Elizabeth University, Glasgow Royal Infirmary, Scotland, UK, E-mail: ce6@doctors.org.uk

***Received:** 11-Feb-2023, Manuscript No. AAASR-23-91017; **Editor assigned:** 14-Feb-2023, PreQC No. AAASR-23-91017(PQ); **Reviewed:** 28-Feb-2023, QC No. AAASR-23-91017; **Revised:** 02-March-2023, Manuscript No. AAASR-23-91017(R); **Published:** 09-Mar-2023, DOI: 10.35841/2591-7765-7.2.137*

***Citation:** Eliot C. Emerging therapeutic strategies for myelodysplastic syndromes: novel targets and approaches. J Adv Surge Res. 2023;7(2):137*

2. Arber DA, Orazi A, Hasserjian R, et al. The 2016 revision to the World Health Organization classification of myeloid neoplasms and acute leukemia. Blood. 2016;127:2391–405.

3. Song Y, Rongvaux A, Taylor A, et al. A highly efficient and faithful MDS patient-derived xenotransplantation model for pre-clinical studies. Nat Commun. 2019;10:366.

4. Steensma DP, Brunner AM, DeZern AE, et al. Low clinical trial accrual of patients with myelodysplastic syndromes: causes and potential solutions. Cancer. 2018;124:4601–9.

5. Sekeres MA, Steensma DP. Rethinking clinical trial endpoints in myelodysplastic syndromes. Leukemia. 2019;33:570–5.

6. Benton CB, Khan M, Sallman D, et al. Prognosis of patients with intermediate risk IPSS-R myelodysplastic syndrome indicates variable outcomes and need for models beyond IPSS-R. Am J Hematol. 2018;93:1245–53.