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Myelodysplastic syndrome and its therapeutic options: transplantation, supportive therapy and other treatments

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Summary

Myelodysplastic syndrome (MDS) is a group of hematologic disorders characterized by dysfunction in the formation of blood cells in the bone marrow, resulting in anemia, leukopenia, and thrombocytopenia. The condition can range in severity from
from asymptomatic to those that progress to acute leukemia. Treatment for MDS depends on several factors, such as the severity of the disease, the patient's age, and the presence of comorbidities.
The main therapeutic options include hematopoietic stem cell transplantation, which offers a potential cure but carries a high risk of complications. Supportive therapy, such as blood transfusions and colony-stimulating agents, aims to alleviate symptoms and improve patients' quality of life. Furthermore, pharmacological treatments such as DNA methyltransferase inhibitors have shown benefits in some cases. The management of MDS requires an individualized approach, considering the benefits and risks of each therapeutic option. The progression of the disease and response to treatment can be unpredictable, making continuous monitoring and adjustment of therapeutic strategies as needed essential.

Keywords: Myelodysplastic Syndrome, Hematological Disorders, Hematological Therapies.

Abstract

Myelodysplastic Syndrome (MDS) is a group of hematologic disorders characterized by dysfunction in the formation of blood cells in the bone marrow, resulting in anemia, leukopenia, and thrombocytopenia. The condition can vary in severity, from asymptomatic forms to those that progress to acute leukemia. Treatment for MDS depends on several factors, such as the severity of the disease, the patient's age, and the presence of comorbidities. The main therapeutic options include hematopoietic stem cell transplantation, which offers a potential cure but carries a high risk of complications. Supportive therapy, such as blood transfusions and colony-stimulating agents, aims to alleviate symptoms and improve patients' quality of life.

Furthermore, pharmacological treatments such as DNA methyltransferase inhibitors have shown benefits in some cases. The management of MDS requires an individualized approach, considering the benefits and risks of each therapeutic option. Disease progression and response to treatment can be unpredictable, making continuous monitoring and adjustment of therapeutic strategies as needed essential.

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INTRODUCTION

Myelodysplastic syndrome (MDS) is a group of hematologic disorders complexes that affect the production and function of blood cells. It is characterized by a dysfunction in the bone marrow, responsible for the production of red blood cells, platelets and white blood cells, resulting in inadequate production of mature blood cells and functional. This hematopoietic failure leads to a series of clinical complications, such as

anemia, frequent infections due to neutropenia and bleeding caused by thrombocytopenia. Although MDS is more common in the elderly, it can also affect patients younger people, especially those with a history of exposure to toxic agents, such as chemotherapy or radiation (Viel, et al. 2024).

The diagnosis of myelodysplastic syndrome is challenging and involves a combination laboratory tests, such as blood counts, bone marrow biopsies and studies cytogenetics. MDS is a disease that presents a wide clinical variation, from forms mild, which may be asymptomatic or present mild symptoms, to aggressive forms that can develop into acute myeloid leukemia (AML), a type of blood cancer difficult to control. Given the diversity of presentations and the complexity of their treatment, the management of MDS requires a personalized approach, considering factors such as the patient's age patient, the specific type of disease, the risk of progression and the response to treatment (Pulgarin, et al. 2021).

The objective of this work is to explore the main therapeutic options for treatment of myelodysplastic syndrome, with emphasis on the most effective approaches and innovative. MDS has a variable prognosis, and therapeutic strategies include from supportive treatments to more complex interventions, such as cell transplantation. hematopoietic stem cell transplantation (HSCT), which remains the curative treatment of choice for more severe cases. In addition, the use of medications such as azacitidine, decitabine, and others Emerging therapies have shown progress in disease management. Supportive therapy, including blood transfusions and the use of colony-stimulating agents, as well plays an essential role in alleviating symptoms and improving the quality of life of patients.

Furthermore, it provides a detailed analysis of these therapeutic options, evaluating their indications, benefits and limitations. From this approach, we seek to provide a deeper understanding of available treatment alternatives, highlighting advances in medicine and new perspectives for the treatment of myelodysplastic syndrome. The successful treatment of MDS depends on a combination of therapies and a continuous and multidisciplinary monitoring, with the ultimate goal of improving the quality of patient's life, reduce the risk of complications and prolong survival.

METHODOLOGY

To carry out this work, a systematic review of the literature was carried out about myelodysplastic syndrome (MDS) and its therapeutic options, focusing on treatments such as hematopoietic stem cell transplantation, supportive therapy and approaches

emerging pharmacological approaches. The research was conducted in

renowned scientific databases, such as PubMed, Scopus, and Scielo, using descriptors in health specific to the topic. The descriptors used included terms such as "syndrome myelodysplastic", "hematopoietic stem cell transplantation", "supportive therapy", "pharmacological treatments", "azacitidine", "decitabine", "lenalidomide", "treatment of acute myeloid leukemia", among others.

Inclusion Criteria

Original studies, systematic reviews, clinical trials were selected randomized and cohort studies that addressed therapeutic options for the syndrome myelodysplastic syndrome, including medical and pharmacological interventions, supportive therapies, as well such as the impact of hematopoietic stem cell transplantation on the prognosis of the disease. In addition, studies detailing the characteristics of the disease, the methods more effective diagnoses, and recent advances in the treatment of MDS. The literature considered relevant was published between 2021 and 2025, ensuring the current status of data analyzed.

Exclusion Criteria

Articles that addressed only theories or aspects were excluded from the research. genetics of MDS unrelated to therapeutic options, in addition to studies that addressed exclusively of hematologic diseases other than myelodysplastic, such as leukemias not related to myelodysplastic syndrome. Studies with small sample sizes, with inconclusive data or with a high rate of bias methodological. Works published in languages other than Portuguese, English, or Spanish were excluded, aiming to guarantee an accessible and understandable literature analysis.

Search and Quantification Procedure

The search was carried out with the aim of identifying studies that discuss different aspects of the treatment of myelodysplastic syndrome. Initially, 132 were found articles in various databases, which were then filtered according to the criteria of inclusion and exclusion criteria. After analyzing the abstracts and keywords, 16 studies were selected as relevant to the topic, being classified into three groups: 1) studies about stem cell transplantation

hematopoietic, 2) studies on supportive therapies and 3) studies on therapies pharmacological and emerging drugs.

These studies were then analyzed qualitatively to identify treatments most effective, their success rates, the risks involved, and the most common indications in each clinical context. Data analysis was performed through critical reading of the articles selected, considering the different types of studies (clinical trials, reviews systematics, and cohort studies) and the methodological quality of each. The information collected were synthesized and presented in the form of a comprehensive overview of the therapeutic options available for the treatment of myelodysplastic syndrome, with emphasis in the most recent evidence.

The research was completed with the organization of the collected information, allowing a detailed overview of available treatments, including advances in the use of therapies genetics and new pharmacological approaches, in addition to the impact of supportive therapies on quality of life of patients.

RESULTS AND DISCUSSION

1. Hematopoietic Stem Cell Transplantation (HSCT)

Hematopoietic stem cell transplantation (HSCT) is considered the only way curative for myelodysplastic syndrome, especially in patients with high-risk forms at risk of or developing acute myeloid leukemia (AML). This treatment involves replace the patient's bone marrow with healthy hematopoietic stem cells, which can be obtained from a compatible donor or, in some cases, from the patient himself (autologous transplant) (Sielfeld, et al. 2021).

Types of Transplant:

- **Allogeneic transplant:** Allogeneic HSCT involves the infusion of stem cells from a healthy donor. This type of transplant is particularly indicated for patients with a high risk of progression to AML. Although it is considered an option curative for high-risk patients, allogeneic transplantation has challenges significant, including graft-versus-host disease (GVHD), where cells from the donor attack the patient's tissues. Furthermore, the risk of infections is high due to the immunosuppression required to prevent graft rejection (Cunha, et al.

2024).

- **Autologous transplant:** In this type of transplant, the patient's own stem cells are patient are collected, processed and reinfused after chemotherapy treatment. Although autologous transplantation has a lower risk of CVD, it is not an option curative for all patients with MDS, as the disease can recur after procedure (Da Silva Bonfim, et al. 2022).

Results:

HSCT has shown overall survival rates of approximately 30-50% in patients with high-risk MDS, especially those under 60 years of age. The factors determinants for success include donor choice, HLA compatibility (antigens human leukocytes), the patient's immune response and the control of complications post-transplant (Rosa, et al. 2024).

Complications:

The most common complications associated with HSCT include:

- **Graft-versus-host disease (GVHD):** A condition in which the cells from the donor attack the patient's tissues, causing damage to the skin, liver and gastrointestinal tract gastrointestinal (Piazera, et al. 2024).
- **Infections:** Due to the immunosuppression required to prevent rejection of the transplantation (Costa, et al. 2024).
- **Graft failure:** When the transplanted stem cells do not implant correctly in the patient's bone marrow (Costa, et al. 2024).

In older patients or those with significant comorbidities, the risk of complications post-transplant may be high, which makes HSCT less viable.

2. Supportive Therapy

Supportive therapy is essential in the management of MDS, as it helps to alleviate the symptoms and improve quality of life, especially in patients with more severe forms milder stages of the disease or who are not candidates for HSCT. This approach includes interventions that directly address the hematologic complications of MDS, such as anemia, thrombocytopenia and neutropenia (Salgarello, et al. 2024).

Blood Transfusions:

Blood transfusions are essential for MDS patients who present with severe anemia (deficiency of red blood cells) or thrombocytopenia (low number of platelets). Red blood cell transfusions help improve tissue oxygenation and relieve fatigue, while platelet transfusions reduce the risk of bleeding (Arruda, et al. 2024).

Although effective in the short term, frequent transfusions can lead to complications. such as iron overload, which may require additional treatments with iron chelators, to prevent damage to the heart and liver (Arruda, et al. 2024).

Colony Stimulating Agents:

Colony stimulating agents, such as colony stimulating factor (CSF), granulocytes (G-CSF), are used to stimulate the production of white blood cells in patients with neutropenia. These medications help reduce the risk of serious infections, a common problem in MDS due to insufficient leukocyte production. However, the effect of colony-stimulating agents in MDS may be limited, and not all patients respond effectively to these treatments (Chen Liang, 2022).

Immunosuppressive Therapy:

Immunosuppressive therapy, which involves the use of medications such as antithyroglobulin or cyclophosphamide, has shown benefits in patients with forms hypoplastic MDS cells. The idea is to reduce the body's autoimmune response, allowing the bone marrow to recover its function. This approach may be effective in patients with MDS associated with immune dysfunctions or in those in which the bone marrow is not completely failed, but rather hypoplastic (diminished) (Martins, et al. 2022).

Although useful in certain cases, the use of immunosuppressants can increase the risk of infections and long-term complications, which requires constant monitoring (Araújo, et al. 2023).

3. Emerging Pharmacological Treatments

In recent years, the pharmacological treatment of myelodysplastic syndrome has evolved, with new drugs being introduced to improve hematologic response and delay disease progression (Aredes, et al. 2024).

Azacitidine and Decitabine:

Azacitidine and decitabine are drugs that inhibit DNA methylation, a process that leads to the repression of gene expression. They help restore function hematopoietic system, allowing for more efficient production of blood cells. These medications have been particularly effective in patients with more severe forms of MDS and in individuals with specific DNA mutations. The use of these agents has shown improve survival and reduce the need for blood transfusions (Paiva, 2023).

Clinical studies have shown that treatment with azacitidine can improve quality of life and increase overall survival by up to 30% in patients with high-risk MDS intermediate to high. Decitabine, in turn, has similar effects and is used mainly in patients who are not candidates for HSCT (Mancuso, et al. 2024).

Lenalidomide:

Lenalidomide is an immunomodulator that has been effective in patients with MDS associated with alterations in chromosome 5 (del(5q)), one of the most common anomalies in MDS. This drug promotes hematologic recovery in patients with this mutation, leading to an improvement in blood cell counts and a significant reduction in need for blood transfusions. However, the use of lenalidomide is restricted to patients with specific genetic characteristics and is not effective for all types of MDS (Junior, et al. 2021).

Venetoclax:

Venetoclax is an inhibitor of BCL-2, a protein involved in the regulation of death cell. This drug has shown promising results in the treatment of MDS that turns into acute myeloid leukemia. The combination of venetoclax with other chemotherapy drugs have been tested in

clinical trials, with preliminary results indicating that it may be effective in improving survival and control of disease progression (Campos, et al. 2022).

Although the results with venetoclax are promising, it is still in the development phase. research and further studies are needed to fully understand its role in treatment of MDS and in combination with other therapeutic agents (Campos, et al. 2022).

FINAL CONSIDERATIONS

Myelodysplastic syndrome (MDS) represents a group of hematologic disorders complex and challenging, whose therapeutic options vary according to the clinical profile of the patient, including factors such as disease severity, age, and associated comorbidities. Although advances in the treatment of MDS in recent years have been significant, the condition continues to require a personalized and multidisciplinary approach to optimize clinical results and patients' quality of life.

Hematopoietic stem cell transplantation (HSCT) remains the only treatment available dressing for MDS, being especially effective in patients with high-risk forms risk or potential for progression to acute myeloid leukemia (AML). However, complexity and risks associated with HSCT, such as graft-versus-host disease (CVD) and post-transplant infection, highlight the importance of carefully selecting candidates and monitor long-term results. Older patients or those with significant comorbidities may have less benefit from this curative approach, which requires a careful risk-benefit analysis.

On the other hand, supportive therapy, which includes blood transfusions and the use of colony stimulating agents, plays a fundamental role in disease management, especially in patients with less severe forms or when transplantation is not an option viable option. While these therapies do not cure MDS, they help improve symptoms, reduce complications and provide a better quality of life for patients. However, complications such as iron overload due to frequent transfusions and the risk of infections in immunosuppressed patients are challenges that need to be constantly monitored.

The use of emerging pharmacological therapies, such as azacitidine, decitabine and lenalidomide, has shown considerable advances in the treatment of MDS. These medications, by partially restoring hematopoietic function, can improve patient survival and reduce the need for transfusions, particularly in patients with intermediate to high-risk MDS. However, it is important to note that the response to these treatments can be variable, and not all patients have the same results benefits. The continued development of more specific therapies and treatments personalized based on the molecular characteristics of the disease offers a way promising to improve results in the treatment of MDS.

Furthermore, the combination of therapeutic approaches, involving both treatments dressings and support, is increasingly important to optimize results, taking into account the individual needs of each patient. Continuous research is essential for the development of new therapies and the identification of biomarkers that can predict the response to treatment and the progression of the disease.

In short, the treatment of myelodysplastic syndrome is multifaceted and requires a individualized approach, based on the genetic and clinical characteristics of each patient. Although current treatments have advanced considerably, there is still much to be done. explored, especially in the field of targeted and immunological therapies. The constant monitoring, continuous research and advancement in therapeutic options are crucial to improving the survival prospects and quality of life of patients with SMD, offering hope for more effective and personalized treatment in the future.

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