aproximadamente 10% a 20% dos pacientes. Nesses casos, o uso precoce de Rituximabe, anticorpo monoclonal anti-CD20, tem sido eficaz, apresentando taxas de resposta clínica entre 74% e 95%. Estudos evidenciam que a combinação do Rituximabe com a terapia padrão aumenta significativamente a recuperação plaquetária, reduz o tempo de hospitalização e diminui a incidência de recidivas, com remissão observada em até 89% dos casos em menos de um mês. Portanto, embora a plasmaférese continue sendo o pilar do tratamento da PTT, é fundamental o reconhecimento precoce da refratariedade para a rápida introdução de terapias imunossupressoras adicionais. O Rituximabe constitui uma opção bem estabelecida para obtenção de resposta clínica sustentada, melhorando o prognóstico e a qualidade de vida dos pacientes.

https://doi.org/10.1016/j.htct.2025.104969

ID – 2493

RECENT ADVANCES IN THE FIELD OF HEMOPHILIA DIAGNOSIS AND TREATMENT

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Introduction: Hemophilia is a rare hereditary bleeding disorder caused by a deficiency of coagulation factors VIII (hemophilia A) or IX (hemophilia B). It is inherited in an X-linked recessive pattern. This condition has been shown to impair the coagulation cascade, which can lead predisposing patients to spontaneous and recurrent bleeding, particularly in joints and muscles. This was a significant impact on the patient's quality of life. **Objectives:** This study is a literature review with the objective of systematizing the extant knowledge regarding the pathophysiology, clinical manifestations, diagnostic methods, and therapeutic strategies of hemophilia. **Material and methods:** A narrative literature review was conducted through searches in the PubMed, SciELO, Web of Science, Scopus, and Google Scholar databases. Publications from the last five years (2020–2025) were prioritized, and those in Portuguese, English and Spanish were included. The following descriptors were utilized: The following terms are relevant to the study: "Hemophilia", "Factor VIII", "Factor IX", "Diagnosis", "Treatment", "Gene therapy", and their equivalents in other languages. **Results:** The underlying pathophysiology of hemophilia is characterized by genetic mutations that impair the production of coagulation factors, particularly factor VIII or IX. Hemophilia A, which accounts for 85% of cases, is the most prevalent form of the condition. Common complications associated with hemophilia A include hemarthroses, severe arthropathies, and the development of inhibitors. Diagnostic methods employed to identify these complications include clinical and laboratory tests such as aPTT, OSCA, and CSA, as well as molecular techniques, including Next-Generation Sequencing (NGS). Treatment has evolved with the advent of plasma-derived and recombinant factor VIII/IX concentrates, as well as novel approaches such as emicizumab (a bispecific antibody), bypassing agents (aPCC and rFVIIa), and gene therapy, which enables sustained expression of factors through viral vectors (AAV). Discussion: Notwithstanding the strides made in the therapeutic realm, persistent, challenges remain, including the exorbitant cost of treatments, the emergence inhibitors and the paucity of diagnostic resources in regions characterized by inadequate infrastructure. The integration of novel technologies, including gene therapy and monoclonal antibodies, has demonstrated considerable potential. However, the effective implementation of these technologies is contingent upon the establishment of comprehensive public policies that ensure equitable access and utilization. **Conclusion:** Although not prevalent, hemophilia poses significant clinical challenges. Advancements in the domains of diagnosis and treatment, encompassing biotechnology and precision medicine, have led to an augmentation in the potential for efficacious management. However, the overcoming structural, economic, and social barriers remains necessary to ensure comprehensive and equitable patients care.

https://doi.org/10.1016/j.htct.2025.104970

ID – 3172

RELATO DE CASO: HEMOFILIA ADQUIRIDA EM PACIENTE COM ARTRITE IDIOPÁTICA JUVENIL

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Introdução: A hemofilia adquirida consiste em uma coagulopatia rara, caracterizada pela formação de autoanticorpos contra o fator VIII, levando a quadros hemorrágicos potencialmente graves, geralmente em pacientes sem histórico prévio de distúrbios hematológicos. Entre os fatores predisponentes, destacam-se doenças autoimunes, neoplasias, infecções e o período pós-parto. Dada à elevada morbimortalidade, o reconhecimento clínico e laboratorial precoce é fundamental,