Opinion Article
Volume 13:2, 2023

Journal of Blood & Lymph

ISSN: 2165-7831 Open Access

Advancements and Ongoing Challenges in Treatment and Management of Hemophilic Factor: Rare Blood Disorder

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Description

A typical hereditary condition called haemophilia impairs the blood's tendency to clot properly. The disorder is caused by a deficiency in one of the blood clotting factors, which are proteins that work together to form a clot and stop bleeding. Hemophilia is caused by a mutation in the genes that control the production of clotting factors. Haemophilia comes in two primary forms: haemophilia A, which is activated on by a lack of clotting factor VIII, and haemophilia B, which is caused on by a lack of clotting factor IX.

Despite being a rare disease, hemophilia can have a significant impact on the lives of those affected by it. Patients with hemophilia experience excessive bleeding and bruising, which can lead to joint damage, chronic pain, and other complications. The management of hemophilia involves the replacement of the deficient clotting factor through regular infusions of clotting factor concentrates. Recent research has led to the development of new drugs that have the potential to revolutionize the treatment of hemophilia. One such drug is emicizumab, which works by mimicking the activity of clotting factor VIII. Emicizumab is administered subcutaneously and has been shown to reduce bleeding episodes in patients with hemophilia A. This drug represents a significant advancement in the treatment of hemophilia, as it offers patients a more convenient and effective treatment option.

However, despite these advancements, there is still much to be learned about hemophilia. Research is needed to better understand the psychological and emotional impact of the disease on patients and their families. Studies have shown that patients with hemophilia often experience social isolation, anxiety, and depression, and more research is needed to develop effective interventions to address these issues. In addition, there is a need for more research on the long-term effects of treatment with clotting factor concentrates. Patients with hemophilia are at increased risk of developing inhibitors, which are antibodies that neutralize the effects of clotting factor concentrates. The development of inhibitors can make it more difficult to manage the disease and can lead to complications such as joint damage and chronic pain. More research is needed to understand the risk factors for inhibitor development and to develop strategies to prevent and treat inhibitors.

Life expectancy varies with disorder severity and access to effective treatment, like many aspects of the disorder. People with severe haemophilia who don't get enough current treatment live much shorter lives and frequently never reach adulthood. The typical life expectancy was only 11 years before the 1960s, when effective treatments became accessible. The average haemophiliac with appropriate care had a 50–60 year life expectancy by the 1980s. With the right care today, males with haemophilia can live nearly normal lives with an average lifetime that is around 10 years less than that of a male without the condition. Since the 1980s, HIV/AIDS acquired through treatment with infected blood supplies has replaced bleeding as the primary cause of mortality in patients with severe haemophilia. Intracranial haemorrhage, which currently accounts for one-third of all fatalities among haemophiliacs, is the second most common cause of death associated with severe complications from haemophilia. Cirrhosis from hepatitis infections and blood flow restriction from soft tissue haemorrhage are two additional leading causes of death.

Hemophilia is a rare genetic disorder that can have a significant impact on the lives of those affected by it. While advancements in the treatment of hemophilia have been made in recent years, there is still much to be learned about the disease. Research is needed to better understand the psychological and emotional impact of the disease on patients and their families, as well as the long-term effects of treatment with clotting factor concentrates.

How to cite this article: Zhong, Qin. "Advancements and Ongoing Challenges Challenges in Treatment and Management of Hemophilic Factor: Rare Blood Disorder." *J Blood Lymph* 13(2023) 302.

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Received: 06-Jun-2023, Manuscript No. JBL-23-101493; **Editor assigned:** 08-Jun-2023, Pre QC No JBL-23-101493 (PQ); **Reviewed:** 22-Jun-2023, QC No JBL-23-101493; **Revised:** 29-Jun-2023, Manuscript No. JBL-23-101493 (R); **Published**: 06-Jul-2023, DOI: 10.37421/ 2165-7831.2023.13.302