This Week in Hemophilia

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Effects and costs of Emicizumab compared to FVIII concentrates

Link: https://onlinelibrary.wiley.com/doi/epdf/10.1002/pbc.31351

This study focused on severe hemophilia A (SHA) in children, a condition where the blood lacks factor VIII (FVIII), a key protein for clotting. This deficiency leads to frequent, sometimes dangerous bleeding episodes, especially into joints. Traditional treatment involves regular infusions of FVIII concentrates (CFC), but this can be burdensome, requiring frequent IV access and increasing risks like infections or vein damage. A newer treatment, emicizumab, offers a more convenient subcutaneous injection. While emicizumab has been widely studied in older children and adults, less is known about how it compares to CFC in younger children without inhibitors, a specific complication where the body rejects FVIII treatment.

The researchers conducted a retrospective self-control study, analyzing medical records of 15 children under 12 years old who switched from CFC to emicizumab. By comparing each child's bleeding history during equal periods on both treatments, they assessed outcomes like annualized bleeding rates (ABR), joint health, and costs. The study avoided potential bias by using the same duration of data collection for both treatment phases.

Results showed that emicizumab significantly reduced bleeding into joints and spontaneous bleeds compared to CFC. However, muscle bleeds were slightly more common and severe with emicizumab, though the difference was not statistically significant. Additionally, emicizumab eliminated complications related to central venous access devices (CVAD), which are commonly needed for CFC therapy. Importantly, emicizumab also cost significantly less, saving an average of nearly $200,000 per patient annually.

These findings underscore emicizumab's potential as a safer, more convenient, and cost-effective alternative for children with SHA. The study highlights that while emicizumab is effective in reducing overall bleeding, attention is needed for muscle bleeds. Long-term studies are essential to understand its full impact on joint health and other outcomes. For families and healthcare providers, this research reinforces emicizumab as a strong option, making hemophilia management less invasive and more accessible, improving quality of life for young patients.

Chronic hepatitis C virus infection and liver disease in men with hemophilia

Link:https://ashpublications.org/bloodadvances/article/8/22/5767/517941/Predictors-of-liver-disease-outcomes-in

This study focused on the connection between chronic hepatitis C virus (HCV) infection and liver disease in men with hemophilia, a bleeding disorder where blood doesn't clot properly. Decades ago, many people with hemophilia were exposed to HCV through contaminated blood products, which led to long-term liver problems. The researchers aimed to identify factors that increase the risk of

severe liver disease (end-stage liver disease or ESLD) and liver cancer (hepatocellular carcinoma or HCC) in this group.

To study this, they reviewed the medical records of 121 men treated at a hemophilia center. These men, aged on average 54 years, had been living with HCV for nearly their entire lives. The team analyzed liver outcomes like ESLD, HCC, and platelet levels—a marker of liver health—using statistical tests to find patterns and predictors.

The results showed that about 20% of the men developed ESLD, and 6% had HCC. Importantly, low platelet levels and co-infection with HIV significantly increased the risk of ESLD. ESLD itself was the strongest predictor of HCC. The good news is that none of the men treated with modern antiviral therapies developed ESLD, underscoring the importance of treating HCV early. However, even after clearing the virus, damage like cirrhosis often remained, leaving patients vulnerable to complications.

These findings highlight the ongoing burden of liver disease in people with hemophilia who contracted HCV. Despite advances in antiviral treatments, many still face high risks of serious liver issues, especially if they are also living with HIV or have low platelet counts. This research stresses the need for regular monitoring of liver health in this population and reinforces the critical role of antiviral therapies in reducing liver disease progression.

For those affected, this study reinforces the importance of staying proactive with liver health checks and discussing antiviral therapy with healthcare providers. It also points to a need for tailored care as individuals with hemophilia and HCV age, to manage long-term risks effectively.

Emicizumab and Acquired Hemophilia A

Link:https://ashpublications.org/bloodadvances/article/doi/10.1182/bloodadvances.2024013882/517940/Real-world-impact-of-emicizumab-amp

Acquired Hemophilia A (AHA) is a rare autoimmune disorder where the body produces antibodies that attack its own Factor VIII, a crucial protein for blood clotting. This leads to severe, spontaneous bleeding that can be life-threatening. Current treatments focus on stopping the bleeding and eliminating the antibodies using immunosuppressive therapies (IST). However, IST carries significant risks, including serious infections. Additionally, existing clotting treatments often require hospitalization and complex monitoring, leaving a gap for effective outpatient care.

This study explored the use of emicizumab, a long-acting medication given by injection, originally designed for congenital hemophilia A. It acts like Factor VIII and helps blood clot without being affected by the antibodies that attack natural Factor VIII. Researchers collected data on 62 patients across 12 U.S. treatment centers who were treated with emicizumab for AHA. Most participants had severe bleeding at diagnosis, and many experienced ongoing bleeding despite standard therapies. Emicizumab was used as a preventive measure to manage bleeding while patients were treated for their underlying condition with IST.

Results showed that emicizumab significantly reduced bleeding events. After starting emicizumab, 87% of patients had no further bleeding episodes, and those who did had manageable breakthrough events. Moreover, emicizumab allowed for more outpatient care, reducing hospital stays and improving quality of life. Nearly 56% of patients achieved complete remission, where bleeding

stopped, and Factor VIII levels normalized. Importantly, adverse events were rare, and no serious bleeding or infections were linked to the treatment.

This study highlights emicizumab as a game-changer for AHA, making outpatient management feasible and safer while reducing the risks associated with IST. It also suggests that combining emicizumab with targeted IST could be the most effective strategy, addressing both immediate bleeding risks and long-term remission. By bridging a critical gap in treatment, emicizumab holds promise for improving outcomes and quality of life for people with this challenging condition.