

# This Week in Hemophilia

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## New Preventive Treatment for HIV

Link: <https://www.nejm.org/doi/full/10.1056/NEJMoa2407001>

HIV has historically been a significant concern for hemophiliacs because blood product contamination posed a high risk of transmission. While developed countries have nearly eliminated this risk through recombinant proteins, a new drug like lenacapavir still offers important benefits. This twice-yearly injection provides a promising prevention option, particularly for those who might struggle with daily medication adherence.

The study examines the effectiveness of twice-yearly subcutaneous injections of lenacapavir compared to daily oral pills of emtricitabine-tenofovir alafenamide (F/TAF) for preventing HIV in cisgender women. The problem being addressed is the low uptake and adherence to daily pre-exposure prophylaxis (PrEP) pills, which limits their effectiveness in preventing HIV, particularly among women. This is a significant issue because consistent and proper use of PrEP is essential to reduce the incidence of HIV infections, which remains a substantial public health challenge globally.

The researchers conducted a phase 3 clinical trial, which is a late-stage study to confirm the effectiveness and safety of a new treatment. They randomly assigned 5,338 participants from South Africa and Uganda to receive either the lenacapavir injection, daily F/TAF pills, or daily emtricitabine-tenofovir disoproxil fumarate (F/TDF) pills. A phase 3 trial typically involves a large number of participants and is designed to gather comprehensive data on the treatment's efficacy and side effects.

Results showed that none of the participants who received lenacapavir acquired HIV, whereas there were 39 infections in the F/TAF group and 16 in the F/TDF group. This means that lenacapavir was significantly more effective at preventing HIV than both F/TAF and F/TDF. The study also noted that adherence to the daily pill regimens was low, which likely contributed to the higher infection rates in those groups. Additionally, lenacapavir had a higher rate of injection-site reactions but no serious safety concerns.

These findings are crucial as they suggest that a twice-yearly injection could be a more effective and convenient option for HIV prevention, particularly for women who may struggle with daily pill adherence. This could lead to better health outcomes and a significant reduction in new HIV infections. By offering a long-acting alternative to daily PrEP, lenacapavir has the potential to improve adherence rates and provide consistent protection against HIV, contributing to the global effort to control the HIV epidemic.

## Long term analysis of prophylaxis in 50+ years old people with hemophilia

Link:

<https://ashpublications.org/bloodadvances/article/doi/10.1182/bloodadvances.2023012462/517123/Long-term-Clinical-Outcomes-of-Prophylaxis-With-a>

Hemophilia is a genetic disorder where blood doesn't clot properly, leading to excessive bleeding. This study focused on older adults (50+ years) with hemophilia A or B who received prophylactic treatment using two extended half-life factor replacements: rFVIII Fc for hemophilia A and rFIX Fc for hemophilia B. The importance of this research lies in its focus on an aging population with hemophilia, who not only deal with their bleeding disorder but also face additional age-related health issues like joint disease and hypertension.

The methods involved analyzing data from previous Phase 3 trials (A-LONG/ASPIRE for hemophilia A and B-LONG/B-YOND for hemophilia B) to evaluate the long-term outcomes of these treatments.

Participants were initially enrolled if they had severe hemophilia and were either on-demand therapy with frequent bleeding events or already on prophylactic treatment. They were then followed over several years to track their bleeding rates, joint health, and overall quality of life.

Results showed that using rFVIII-Fc and rFIX-Fc significantly reduced the annualized bleeding rates (ABRs) in these older adults, even in those who had joint issues at the start of the study. Joint health improved, with many participants seeing their joint problems resolved. This is crucial because joint damage from repeated bleeds can be debilitating. The study also found improvements in the quality of life for participants, particularly in physical health and the ability to engage in sports and leisure activities. This indicates that better bleed management allowed these individuals to lead more active and fulfilling lives.

The big picture takeaway is that extended half-life treatments like rFVIII-Fc and rFIX-Fc are effective in managing hemophilia in older adults, providing long-term benefits regardless of the patient's age or the presence of joint issues. This supports the idea that starting and maintaining prophylactic treatment early and consistently can lead to significant health improvements, reducing both the physical and psychological burdens associated with hemophilia and aging. Future research will be essential to develop best practices for managing hemophilia as patients age, considering the additional health challenges they face.

### **The burden of hemophilia B in the United States**

Link: <https://onlinelibrary.wiley.com/doi/epdf/10.1111/hae.15077>

This study focuses on understanding the real-world burden of hemophilia B, a genetic disorder that affects blood clotting, in the United States. Hemophilia B leads to severe bleeding, particularly in joints and muscles, causing long-term damage and pain. Currently, people with severe hemophilia B often use intravenous factor replacement therapy to manage their condition, but new treatments are being explored. This research is crucial because it sheds light on how hemophilia B affects daily life and health care needs beyond controlled clinical trials.

The researchers conducted a retrospective cohort study using data from PicnicHealth, a platform that collects and digitizes medical records from various health care providers. This approach allowed them to compile a comprehensive dataset of patients' medical histories, including details on bleeding rates, comorbid conditions, and treatment regimens. They identified male patients with hemophilia B who had medical records available between April 2015 and September 2020. The study excluded patients who had inhibitors (antibodies that complicate treatment), were enrolled in clinical trials, or used certain bypassing agents.

The study included 75 patients, out of which 48 had data on their annual bleeding rates (ABR). The average age was 21 years, and most were receiving prophylactic (preventive) treatment. The results showed that 96% of patients with severe hemophilia B and 90% of those with mild to moderate hemophilia B experienced at least one bleeding event. Patients with severe disease had a higher average ABR compared to those with milder forms. The most common associated conditions were joint disease (arthropathy), inflammation of the joint lining (synovitis), mental health issues, and diseases of the circulatory system. Pain medication was widely used, with acetaminophen being the most common, followed by celecoxib.

The findings highlight that despite the use of prophylaxis, bleeding episodes and associated complications remain prevalent among patients with hemophilia B. This underscores the need for improved treatment strategies and greater use of prophylaxis to prevent joint damage and enhance the quality of life for patients. This study provides valuable insights into the daily challenges faced by individuals with hemophilia B, emphasizing the importance of continuous advancements in treatment options.

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