

Overcoming the Hurdles: Advances in the Management of Hemophilia



Dear Colleague:

Thank you for your recent participation in the CE activity “Overcoming the Hurdles: Advances in the Management of Hemophilia” with Drs. Craig Kessler and Guy Young, developed by the Annenberg Center for Health Sciences. As you continue to advance the care you provide to these patients, here are the key concepts for you to consider:

- The Medical and Scientific Advisory Council and World Federation of Hemophilia recommend that prophylaxis should be:
 - Initiated at an early age, ideally before age 3 years and prior to the second joint bleed.
 - Individualized and sufficient to prevent all bleeds at all times.
- The treatment of patients with hemophilia is a balancing act comprised of safety, efficacy, and patient adherence to treatment.
- The optimal therapy may vary among individuals with hemophilia, thus shared decision making is essential to understand the patient’s values, goals, and concerns.
- Beyond clotting factor concentrates, several classes of novel therapies are available to treat persons with hemophilia.
 - All are very effective in reducing bleeding compared to clotting factor concentrates.
 - Key characteristics include:
 - Factor VIII mimetics (emicizumab and Mim8*)
 - Administered subcutaneously.
 - Thrombotic complications: emicizumab (infrequent), Mim8 (none reported).
 - Rebalancing agents (concizumab*, fitusiran*, marstacimab*)
 - Administered subcutaneously.
 - Thrombotic complications: concizumab (infrequent), fitusiran (low incidence), marstacimab (none reported).
 - Gene therapies (etranacogene dezaparvovec, fidanacogene elaparvovec, valoctocogene roxaparvovec).
 - Phenotypic improvement in bleeding rate.

*Not approved by the US Food and Drug Administration for hemophilia

We invite you to participate in other accredited activities we offer (www.Annenberg.net). Thank you.

Regards,

The Annenberg Center Team