



Evidence-based Multidisciplinary Hemophilia Management in Treatment Centers and Underserved Community Referral Networks

Wednesday, December 11, 2024

Registration / Sign-In:

06:00 PM – 06:30 PM

Live Meeting with Presentation:

06:30 PM – 07:30 PM

East Central Florida ONS Chapter (ECFONS)

RoseVilla Southern Table

Private Room

43 W Granada Blvd

Ormond Beach, FL 32174

FACULTY PRESENTER

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RSVP Here

Agenda

Recent Advances and Limitations of Factor Replacement Therapy

- Advances in clotting factor replacement therapy
 - Next-generation extended half-life (EHL) products: protein engineering for best-in-disease efficacy; advantages/limitations of EHL products
 - Efanesoctocog alfa: von Willebrand factor–engineered protein conjugate; difference in protein-engineered factor from other EHL products
 - XTEND-1 and XTEND-Kids studies: efanesoctocog alfa prophylaxis in severe hemophilia A
 - Other novel investigational clotting factors
- Navigating the treatment journey
 - Establishing communication and workflows with a hemophilia treatment center
 - Resources for patients and caregivers

Targeting the Coagulation Cascade

- Physiology of the natural anticoagulation pathway
 - Paradoxical thrombotic risk in patients with low natural anticoagulants
 - Restoring hemostatic balance
 - Challenges in targeting anticoagulant pathways

Provided by Clinical Care Options, LLC

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- Rationale for targeting anticoagulant pathways
 - Advantages and challenges
- Emerging therapeutics for clinical practice
- Antithrombin inhibition
 - Fitusiran: siRNA technology
 - Mechanism of action
 - ATLAS-INH and ATLAS-A/B phase III trials in hemophilia A or B with or without inhibitors
 - ATLAS-PPX phase III trial in hemophilia A or B with or without inhibitors who previously received factor or bypassing agent prophylaxis
 - ATLAS-NEO phase III trial in males older than 12 years of age who switched from prior treatment
- Inhibitors of coagulation inhibitors
 - Anti-TFPI (concizumab, marstacimab, MG1113)
 - Mechanism of action
 - Phase III Explorer trials (7, 8, and 10) of concizumab in hemophilia A or B with or without inhibitors
 - Phase III trials of marstacimab in severe hemophilia A or B
- Activated protein C inhibitor (SerpinPC)
 - Mechanism of action
 - Phase II PRESent-2 study

Emerging Substitution Therapy

- Rationale for FVIII mimetic bispecific antibodies
- Currently approved antibody: emicizumab
 - Current challenges and limitations
- Next-generation FVIII mimetic antibody: Mim8/denecimig)
 - Mechanism of action
 - Potency compared with emicizumab
 - Peak thrombin levels
 - Available data: FRONTIER trials
- Considerations for applying novel therapies in clinical practice
 - Individualizing care

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