



Day 1 The National Landscape and HTC Professional Organizations

Governmental Partners

HRSA

CDC

HTC Professional Organizations

ATHN

NHPCC

Hemophilia Alliance

Functioning and Oversight & The Business of Running a Center

Medical Organization and Oversight: Independent vs. Institutionally-embedded HTCs

The Business of Running a Center and the 340B Pharmacy Programs

Patient Advocacy Groups & Community Voices

HFA

Shared Decision Making

NHF

MASAC

Guidelines and Important Achievements

Comprehensive Clinic and Model of Care
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Comprehensive Care - Part 1

Integrated Approach to Disease Management
Levels of Severity
Aging
Women with Hemophilia / Hemophilia in People Who Have the Potential to Menstruate
Surgery Planning and Management

Laboratory Pitfalls

Inhibitor Testing
Inhibitor Surveillance
Disorders that Are Missed Without a Deeper Understanding of Laboratory Testing

Comprehensive Care - Part 2

Ultrasound and Joint Health
Shared Decision Making and Motivational Interviewing

Hemophilia Cases and Management: Answers to Clinical Dilemmas You Can't (or Shouldn't) Find on Google

Patients on Emicizumab with Breakthrough Bleeding
Attendees Bring Challenging Cases for Panel Review

VWD Overview

History of VWD
Physiology of VWF and Control by Shear and ADAMTS13
Subtypes of VWD
Genetics of VWD and Genetic Regulation of VWF

Diagnostic Evaluation of Patients

Bleeding Scores
Laboratory Evaluation of VWD (including NHF/ASH/ISTH guidelines and intro to genetic testing)
Pitfalls of Phenotype Testing

Therapeutic Agents

DDAVP: Medicine and Trials
VWF Concentrates (plasma derived and recombinant)
Adjuvant Therapies (hormones, antifibrinolytics)

Surgical Management

Surgical Management

Special issues

Reproductive issues: Menorrhagia; Pregnancy and Iron Deficiency; Iron Deficient Anemia
Models of Care: Women's Clinic, Girls Clinic, Clinic for People Who Have the Potential to Menstruate
Other Special Populations: Minority Populations (African-American heritage); Amish Population D1472H Variant, H817Q and Low FVIII
Complications of Therapy and Inhibitor Development
Acquired VWD

Case Examples and Therapy Approaches

Novel Therapies: Emicizumab

Loading Dose, Maintenance, and Reloading Dose
Important Laboratory Considerations
Use in Unusual Circumstances
Real World Data and Experience

Gene Therapy

Patient Interview
Background and Overview
Trial Data
Patient Preparedness
Site Preparedness

Novel Therapies: Therapeutic Approaches in Trials

Overview of Novel Approaches in Clinical Trials
Overview of Tissue Factor Pathway Inhibitor
Other Non-factor Therapies in the Pipeline

Introduction To Clinical Research

Overarching Questions: How Does Research Help Advance Patient Care?
Types of Studies, Including: Registries Collaborative Trials, Pharmaceutical (Phase I – IV), IIS, Exploratory Analyses, and Case Reports

Clinical Research

Data Management Considerations
National Blueprint
Vulnerable Populations
Responsibilities of a Principal Investigator
Designing a Study: Protocol Development (IIS and other studies)
Quality, Compliance, and GCP
NIH and Other Grant Opportunities
Industry-sponsored Clinical Trials How to Interact With CROs How to Determine Budgetary Needs

Rare Disorders

Most Frequent Rare Disorders
Acquired Inhibitors

Case Studies, Clinical Management, and Resources

Conclusion: Bringing It All Together
