The Basics of Hemophilia

Nursing Working Group
National Hemophilia Foundation
The National Hemophilia Foundation is dedicated to finding the cures for inherited bleeding disorders and to preventing and treating the complications of these disorders—through education, advocacy, and research.
Hemostatic System

- Blood vessels
- Platelets
- Plasma coagulation system
- Proteolytic or Fibrinolytic system
How Bleeding Stops

- Vasoconstriction
- Platelet plug formation
- Clotting cascade activated to form fibrin clot
Normal Hemostasis

Types of Bleeding Disorders

• Hemophilia A (factor VIII deficiency)
• Hemophilia B (factor IX deficiency)
• von Willebrand Disease (vWD)
• Other
What is Hemophilia?

- Hemophilia is an inherited bleeding disorder in which there is a deficiency or lack of factor VIII (hemophilia A) or factor IX (hemophilia B)
Inheritance of Hemophilia

- Hemophilia A and B are X-linked recessive disorders
- Hemophilia is typically expressed in males and carried by females
- Severity level is consistent between family members
- ~30% of cases of hemophilia are new mutations
Detection of Hemophilia

• Family history

• Symptoms
  – Bruising
  – Bleeding with circumcision
  – Muscle, joint, or soft tissue bleeding

• Hemostatic challenges
  – Surgery
  – Dental work
  – Trauma, accidents

• Laboratory testing
Degrees of Severity of Hemophilia

- Normal factor VIII or IX level = 50-150%
- Mild hemophilia
  - factor VIII or IX level = 6-50%
- Moderate hemophilia
  - factor VIII or IX level = 1-5%
- Severe hemophilia
  - factor VIII or IX level = <1%
U. S. Incidence of Hemophilia

- **Hemophilia A**: 20.6 per 100,000 males
  - Severe: 50-60%

- **Hemophilia B**: 5.3 per 100,000 males
  - Severe: 44%
Types of Bleeds

- Joint bleeding - hemarthrosis
- Muscle hemorrhage
- Soft tissue
- Life threatening-bleeding
- Other
Joint or Muscle Bleeding

• Symptoms
  – Tingling or bubbling sensation
  – Stiffness
  – Warmth
  – Pain
  – Unusual limb position
Life-Threatening Bleeding

- **Head / Intracranial**
  - Nausea, vomiting, headache, drowsiness, confusion, visual changes, loss of consciousness

- **Neck and Throat**
  - Pain, swelling, difficulty breathing/swallowing

- **Abdominal / GI**
  - Pain, tenderness, swelling, blood in the stools

- **Iliopsoas Muscle**
  - Back pain, abdominal pain, thigh tingling/numbness, decreased hip range of motion
Other Bleeding Episodes

- Mouth bleeding
- Nose bleeding
- Scrapes and/or minor cuts
- Menorrhagia
Complications of Bleeding

• Flexion contractures
• Joint arthritis / arthropathy
• Chronic pain
• Muscle atrophy
• Compartment syndrome
• Neurologic impairment
Treatment of Hemophilia

- Replacement of missing clotting protein
  - On demand
  - Prophylaxis
- DDAVP / Stimate
- Antifibrinolytic Agents
  - Amicar
- Supportive measures
  - Icing
  - Immobilization
  - Rest
Factor VIII Concentrate

- Intravenous infusion
  - IV push
  - Continuous infusion
- Dose varies depending on type of bleeding
  - Ranges from 20-50+ units/kg. body weight
- Half-life 8-12 hours
- Each unit infused raises serum factor VIII level by 2 %
Factor IX Concentrate

- Intravenous infusion
  - IV push
  - Continuous infusion
- Dose varies depending on type of bleeding
  - Ranges from 20-100+ units/kg. body weight
- Half-life 12-24 hours
- Each unit infused raises serum factor IX level by 1%
History of Clotting Factor Concentrates

Prior to 1950: whole blood
1952: Hemophilia A distinguished from B
1950-1960: FFP and Cryoprecipitate
Early 1970s: Commercial plasma-derived factor concentrates
Mid-late 1970’s: Home infusion practices
1981: First AIDS death in the Hemophilia community
History of Clotting Factor Concentrates (cont’d)

Mid-1983: Factor concentrates heat treated for hepatitis
1985: All products heat treated for viral inactivation
1987: Monoclonal factor concentrates
1992: Recombinant factor VIII
1994: Recombinant factor IX-albumin free
2001: 2nd generation recombinant factor VIII
Infusions of Factor Concentrates

- Verify product with physician order.
- Dose may be +/- 10% ordered.
- Do not waste factor even if the dose is not exactly what is ordered.
- Reconstitute factor per package insert.
- Infusion rate per package insert or pharmacy instructions.
- Document lot number, expiration date, time of infusion, and exact dose given in units.
Prophylaxis

• Scheduled infusions of factor concentrates to prevent most bleeding
• Frequency: 2 to 3 times weekly to keep trough factor VIII or IX levels at 2-3%
• Types
  – primary prophylaxis
  – secondary prophylaxis
• Use of IVAD necessary in some patients
DDAVP (Desmopressin acetate)

• Synthetic vasopressin
• Method of action -
  – release of stores from endothelial cells raising factor VIII and vWD serum levels
• Administration -
  – Intravenous
  – Subcutaneously
  – Nasally (Stimate)
• Side effects
Stimate

• How supplied
  – 1.5 mg./ ml (NOT to be confused with DDAVP nasal spray for nocturnal enuresis)
  – 2.5 ml bottle - delivers 25 doses of 150 mcg.

• Dosing
  – Every 24-48 hours prn
  – <50 kg. body weight - 1 spray (150 mcg.)
  – >50 kg. body weight - 2 sprays (300 mcg.)
Amicar
(epsilon amino caproic acid)

- Antifibrinolytic
- Uses
  - Mucocutaneous bleeding
- Dosing: 50 - 100 mg./kg. q. 6 hours
- Side effects
- Contraindications
  - Hematuria
Complications of Treatment

- Inhibitors/Antibody development
- Hepatitis A
- Hepatitis B
- Hepatitis C
- HIV
Inhibitors

• Definition
  – IgG antibody to infused factor VIII or IX concentrates, which occurs after exposure to the extraneous VIII or IX protein.

• Prevalence
  – 20-30% of patients with severe hemophilia A
  – 1-4% of patients with severe hemophilia B
Hepatitis

- **Hepatitis A** - small risk of transmission
  - Vaccination recommended

- **Hepatitis B** - no transmissions since 1985
  - Vaccination recommended

- **Hepatitis C** - no transmissions since 1990
  - ~90% of patients receiving factor concentrates prior to 1985 are HCV antibody positive
Human Immunodeficiency Virus

- No transmissions of HIV through factor concentrates since 1985 due to viral inactivation procedures

- HIV seropositive rate -
  - 69.6% of patients with severe hemophilia A receiving factor concentrates prior to 1985
  - 48.6% of patients with severe hemophilia B receiving factor concentrates prior to 1985
Nursing Considerations

- Factor replacement to be given on time
- Laboratory monitoring
- Increase metabolic states will increase factor requirements
- Factor coverage for invasive procedures
- Document - infusions, response to treatment
- Avoid NSAIDS
- Utilize Hemophilia Center staff for questions / problems
Psychosocial Issues

- Guilt
- Challenge of hospitalizations
- Control issues
- Financial / insurance challenges
- Feeling different / unable to do certain activities
- Counseling needs
Hemophilia Treatment Center Team Members

- Patient / Family
- Hematologist
- Nurse
- Social Worker
- Physical Therapist
- Orthopedist
- Primary Care
- Infectious Disease
- Genetics
- Pharmacy
- Dental
- Hepatology
Role of Hemophilia Treatment Centers

- State-of-the-art medical treatment for persons with hemophilia through the life span
- Education
- Research
- Outreach
- Model of comprehensive care for chronic disease