Bleeding Disorders 101

Women in Government

4th Annual Healthcare Summit

Amy MacDougall, RN, BSN
Senior Clinical Specialist
Baxter Healthcare Corporation
Decisions that will impact future generations of hemophilia
Objectives

• Review the definition, genetics and types of hemophilia
• Discuss the consequences of bleeding
• Describe benefits to hemophilia patient centered comprehensive care
• Advance state approaches to hemophilia
Hemophilia is a rare inherited blood clotting disorder characterized by prolonged bleeding\(^1,2\)

- An estimated 20,000 patients with hemophilia in the United States, almost exclusively male\(^3\)
- Affects all races and ethnic groups\(^1\)
- Occurrence: 1:5000 live male births\(^2\)
- ~400 babies are born with hemophilia each year\(^2\)

- About two-thirds of patients have a known family history of disease\(^3\)
- Chronic disease typically diagnosed as infant with no cure\(^4\)
- Bleeding can be painful, impair normal functions, damage organs or become life-threatening or fatal\(^5\)

**Sources:**
Hemophilia Genetics: Female Carrier

Hemophilia Genetics: Affected Male

Father with Hemophilia  
Normal Mother

Normal  
Carrier  
Person w/ Hemophilia

Normal  
Normal  
Carrier  
Carrier

Two types: Hemophilia A and Hemophilia B

- Two types of hemophilia, where patient has deficiency of clotting factor protein
  - Hemophilia A: Factor VIII
  - Hemophilia B: Factor IX

- 1 in 5,000 male babies are born in the US with hemophilia A each year.
- 1 in 25,000 male babies are born with hemophilia B.

A person with hemophilia does not bleed faster than someone without hemophilia—they just bleed longer.²

### Severity and Bleeding Patterns

<table>
<thead>
<tr>
<th>Mild</th>
<th>Moderate</th>
<th>Severe</th>
</tr>
</thead>
<tbody>
<tr>
<td>• &gt;5% - &lt;40% of normal factor</td>
<td>• 1 - 5% of normal factor</td>
<td>• &lt;1% of normal factor</td>
</tr>
<tr>
<td>• Rare spontaneous bleeding</td>
<td>• Occasional spontaneous bleeding</td>
<td>• Spontaneous bleeding into joints or muscles</td>
</tr>
<tr>
<td>• Bleeding occurs with major trauma or surgery</td>
<td>• Prolonged bleeding with minor trauma or surgery</td>
<td></td>
</tr>
</tbody>
</table>

What does bleeding look like?

Photo courtesy of the National Hemophilia Foundation.

Bruising

• Mid bruise stage

• Late bruise stage

Photos Courtesy of Leonard Valentino, MD. Copyright Rush University Medical Center 2013.
History of Hemophilia Treatment¹⁻³

Safety Evolution

1970s
- Early 1970s: Plasma concentrates available
- Late 1970s: Donor/plasma screening for HBV

1980s
- Mid 1980s: Heat-treated plasma concentrates
- Late 1980s: Enhanced viral inactivation, expanded donor screening

1990s
- Early 1990s: Recombinant FVIII (rFVIII)
- Late 1990s: Improved donor/plasma screening; HIV HCV NAT starts

2000s
- Early 2000s: rFVIII free of blood-based additives

What is factor replacement therapy?¹²

- Replaces missing factor protein
- Is given through a vein
- Two kinds of factor
  - Plasma-derived
  - Recombinant (MASAC recommendation)
- Goal of factor therapy is to stop, if not prevent, the bleeding

Physician or HTC oversees guidance on factor therapy
Medical and Scientific Advisory Council (MASAC) defines quality care in hemophilia

- Regarded as the clinical expert leader that establishes quality of care guidelines for treatment of hemophilia and other bleeding disorders

- MASAC guidelines for standards of care include:
  - Comprehensive care through hemophilia treatment centers (HTCs) (coordinated care)
  - Factor replacement therapy with recombinant factor VIII
  - Prevention of bleeds

Quality Hemophilia Care: Coordinated care through hemophilia treatment centers (HTCs)\(^1\)

- Coordinated care has demonstrated improved lives of patients with hemophilia A and is the standard of care\(^1-3\)
- 70\%-80\% of people with hemophilia rely on HTCs for managing their condition\(^4\)
- CDC and MCHB supports a network of 141 federally-funded HTCs\(^5\)

**High-Touch, Multi-Disciplinary Approach\(^1,5\)**

<table>
<thead>
<tr>
<th>Social Worker</th>
<th>Geneticist</th>
</tr>
</thead>
<tbody>
<tr>
<td>Orthopedist</td>
<td>Dentist</td>
</tr>
<tr>
<td>Nurse Coordinator</td>
<td>Pediatrician</td>
</tr>
<tr>
<td>Physical Therapist</td>
<td>Hematologist</td>
</tr>
</tbody>
</table>

**Patient**

---

**Sources:**
HTC-based care has been associated with better patient outcomes

Five-year study of outcomes from 11 federally-funded comprehensive hemophilia centers\textsuperscript{15}

<table>
<thead>
<tr>
<th>Measure</th>
<th>Reduction</th>
<th>Percent Reduction</th>
</tr>
</thead>
<tbody>
<tr>
<td>Overall costs /Patient /year</td>
<td>$15,800 to $5,932\textsuperscript{*}</td>
<td>62%</td>
</tr>
<tr>
<td>Average hospital admissions / year</td>
<td>from 1.9 to 0.26</td>
<td>86%</td>
</tr>
<tr>
<td>Average days / year as inpatient</td>
<td>from 9.4 to 1.8</td>
<td>81%</td>
</tr>
<tr>
<td>Unemployed Adults</td>
<td>from 36% to 13%\textsuperscript{**}</td>
<td>64%</td>
</tr>
</tbody>
</table>

\textsuperscript{*}This figure represents a retrospective estimate from a small sample, in the case of most centers.

\textsuperscript{**}Based on status at intake interview or annual visit during specified year divided by number of patients seen in that year.

Multivariant analysis of 2950 males with hemophilia in 6 US states between 1993-1995\textsuperscript{16}

\textbf{40\% Reduction in the risk of Mortality}

- People receiving care at HTC
- People not receiving care at HTC

\textbf{P=0.002}

CI=0.5-0.8  RR=0.6

MASAC recommends open access to all hemophilia clotting factor therapies

- Clotting factor therapies
  - Neither pharmacologically nor therapeutically equivalent
  - Vary based upon purity, half-life, recovery, method of manufacture, viral removal and inactivation processes, potential immunogenicity, and other attributes

- Product choice should be agreed upon by patient and healthcare provider
  - Characteristics of each product require complex decision making

“Benefit of limiting products to one within a class, such as one recombinant factor VIII concentrate, solely for the purpose of cost containment is not supported by present clinical practice or by published data.”

State Approaches to Hemophilia

• **Medicaid**
  - Fee for Service
    - Carved Out
  - Medicaid Managed Care
    - Carved Out
    - Stop Loss / Risk Adjustment
  - Disease Management Programs
    - Specific Utilization Programs for Beneficiaries with Hemophilia

• **Private Insurance**
  - Protection from Out of Pocket (Tiering) Discrimination
• **Hemophilia** is a rare, chronic genetic disorder

• **Hemophilia** is well-managed with predictable costs, due to a federally funded coordinated care model and advancements in hemophilia therapy and treatment

• **Hemophilia** has been recognized by state public policy makers as unique with Medicaid and Private Insurance protections
For More Information Contact:

Valery E. Gallagher
Director, US State Government Affairs
Baxter Healthcare
valery_gallagher@baxter.com
224.948.3608