Cost of Care in Hemophilia and Possible Implications of Health Care Reform

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OBJECTIVES

- Introduction
- Health Care Reform
  - Implications for Providers and Consumers
- Cost of Care in Hemophilia
  - How it is measured
  - What we know
  - Limitations of current literature
  - Health care reform issues
INTRODUCTION
Overall Trends in Health Care

- Evidence based medicine and guidelines- what works, what doesn’t
- Increased proportion of health care financed by government so increasing scrutiny and concern
- Use of comparative effectiveness analysis to determine relative benefit of different treatment options
- Health care reform aims to transform how health care is delivered and how services are paid to promote value and reward high quality of care
Small population impacted but relatively large health care expenditures; chronic condition; requires life long treatment;

Appropriate care can lead to reduced morbidity and mortality; no standard treatment guidelines

Limited resources for health care; can become target of cost cutting efforts; health plans with few individuals impacted may be unaware of average/usual costs
### Health care reform timeline and implications for consumers

#### Consumers

| 2010 | • Prohibits insurance companies from denying coverage to children under 19 years of age due to a pre-existing condition  
• Prevents plans from rescinding coverage  
• Eliminates lifetime dollar limits on insurance coverage for essential benefits (like hospitalization)  
• Requires coverage for certain preventive services without deductible, co-pay or coinsurance  
• Regulates use of annual dollar limits on amount of insurance coverage an individual may receive  
• Provides relief for Medicare seniors who hit the prescription drug donut hole (after spending $2,800-responsible for full costs until $4,550 is reached)  
• Provides insurance for uninsured Americans with pre-existing conditions  
• Extends coverage for young adults (on parents plan until turn 26 years old) and early retirees (between ages of 55–65)  
• Increases number of people on Medicaid |

| 2011 | • Provides prescription drug discounts for Medicare seniors  
• Covers annual wellness visits and personalized prevention plans for seniors on Medicare  
• Allows states to offer home and community based services to disabled individuals through Medicaid rather than institutional care in nursing homes (Oct 2011) |
### Health care reform timeline and implications for consumers

<table>
<thead>
<tr>
<th>Year</th>
<th>Events</th>
</tr>
</thead>
</table>
| 2012 | • Aims to understand and **reduce health disparities**  
      • Provides for voluntary **options for long-term care insurance for adults who become disabled** |
| 2013 | • **Improves preventive health coverage**  
      • Additional **funding for Children’s Health Insurance Program (CHIP)** |
| 2014 | • **Prohibits discrimination** due to preexisting conditions or gender  
      • **Eliminates annual limits** on insurance coverage  
      • Provides **tax credits** for middle class to afford insurance  
      • Provides **health insurance exchanges** for those who do not have insurance coverage  
      • **Expands eligibility for Medicaid**  
      • **Requires** individuals to obtain **insurance** if they can afford it  
      • Uses employer insurance funds to purchase insurance through **insurance exchanges** |
## Health care reform timeline and implications for health care providers

<table>
<thead>
<tr>
<th>Year</th>
<th>Actions</th>
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</table>
| 2010 | • Cracks down on fraud and waste in Medicare, Medicaid and CHIP (new screening tools for health care providers)  
• Expands primary care workforce  
• Increases payments for rural health providers  
• Strengthens and expands community health centers and expand preventive and primary health care services at existing sites |
| 2011 | • Establishes Center for Medicare & Medicaid Innovation (CMMI)–to test new ways of delivering care to patients, and national strategy to improve quality in health care  
• Mandates care transitions for seniors (when they leave the hospital) to avoid unnecessary readmissions by coordinating care and connecting patients to services in the community  
• Mandates that 85% (large group) or 80% (small group) of all premiums are spent on health care services and health care quality improvement |
# Health care reform timeline and implications for health care providers

## Health care providers

### 2012
- **Links payment to quality outcomes (value-based purchasing)** in Medicare (hospital performance publicly reported) starting with heart attacks, health failure, pneumonia, surgical care, health-care associated infections and patients’ perceptions of care (after Oct 2012)
- Encourages **Accountable Care Organizations** (physicians join together to coordinate patient care, improve quality, prevent disease and illness and reduce unnecessary hospital admissions)
- Mandates **reduction of paperwork** and administrative costs (standardize billing and implement electronic health information exchange) (Oct 2012)

### 2013
- **Bundles payments** to encourage hospitals, doctors and other providers to work together to improve the coordination and quality of patient care—flat rate payment for episode of care
- Increases Medicaid payments for primary care doctors

### 2014
- Ensures **coverage for individuals participating in clinical trials** for cancer or other life-threatening diseases
- Provides small business **tax credit** (for contribution to provide health insurance for employees)

### 2015
- Pays physicians **based on value not volume** (higher payments for those providing higher value or quality care)
Cost of Illness
Hemophilia Utilization Group Study (HUGS)
Key Findings

HUGS Va – (Hemophilia A)
Data collection complete
HUGS Vb (Hemophilia B) – In progress

HUGS Va funded by CSL Behring, Baxter Health Care Corp, Bayer Foundation, Novo Nordisk and Pfizer (formerly Wyeth)
10 PARTICIPATING HEMOPHILIA TREATMENT CENTERS in HUGS - Evaluating Cost of Care and Burden of Illness in Persons with Factor VIII (IX) Deficiency

HUGS Va and Vb

- Children's Hospital of Orange County, CA
- Children’s Hospital Los Angeles, CA
- University of Colorado Hemophilia & Thrombosis Center, CO
- Indiana Hemophilia & Thrombosis Center, IN
- New England Hemophilia Center, MA
- Gulf States Hemophilia and Thrombophilia Center, TX

HUGS Vb – Additional Centers

- Akron Children’s Hospital, OH
- Puget Sound Blood Center, WA
- Michigan State University, MI
- University of Mississippi Medical Center, MS
DATA COLLECTION OVER 2 YEARS

- Age between 2 to 64 years
- Receiving 90% of their hemophilia care at the participating comprehensive HTCs

<table>
<thead>
<tr>
<th>Initial Interview</th>
<th>Follow-up Surveys</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Participant questionnaire</strong></td>
<td><strong>Participant questionnaire</strong></td>
</tr>
<tr>
<td>- Socio-demographics</td>
<td>- Missed work/school</td>
</tr>
<tr>
<td>- Insurance</td>
<td>- Caregiver support</td>
</tr>
<tr>
<td>- Access to care</td>
<td>- Treatment pattern over time</td>
</tr>
<tr>
<td>- Hemophilia treatment pattern</td>
<td>- Bleeding episodes</td>
</tr>
<tr>
<td>- Other medical conditions</td>
<td>- Health status/quality of life</td>
</tr>
<tr>
<td>- Health status/quality of life</td>
<td></td>
</tr>
<tr>
<td><strong>Clinical information</strong></td>
<td><strong>Clinical information</strong></td>
</tr>
<tr>
<td>- Height, weight</td>
<td>- Healthcare service utilization at HTC</td>
</tr>
<tr>
<td>- History of inhibitor and current inhibitor</td>
<td>- Range of motion</td>
</tr>
<tr>
<td>- Hemophilia treatment pattern</td>
<td>- Hospitalization/Emergency Room visits</td>
</tr>
<tr>
<td>- Range of motion</td>
<td><strong>Factor dispensing information</strong></td>
</tr>
</tbody>
</table>

Monthly
DISTRIBUTION OF PARTICIPANTS BY HEMOPHILIA SEVERITY¹

- HUGS Va – Hemophilia A (N=329)
MOST CHILDREN AND ADULTS HAVE SOME FORM OF HEALTH INSURANCE – Majority Have Private Insurance

HUGS Va

**Children (N=165)**

- Public: 31%
- Private: 62%
- Both public & private: 7%
- No Insurance: 0%

**Mean out of pocket monthly premium = $208.90**
(Range: 0 – 2000)

**Adults (N=164)**

- Public: 33%
- Private: 54%
- Both public & private: 6%
- No Insurance: 7%

**Mean out of pocket monthly premium = $156.20**
(Range: 0 – 1273)
Who are those without insurance?

- HUGS Va:
  - Twelve adults without insurance
  - Hemophilia severity: Severe (n=7), mild (n=4), moderate (n=1)
  - All 12 adults are not on prophylaxis
| MOST FREQUENT CHOICES PARTICIPANTS OR PARENTS MADE BECAUSE OF HEMOPHILIA A TO GET OR MAINTAIN HEALTH INSURANCE³ |
|---|---|
| | HUGS Va |
| | Parents (N=165) | Adults (N=164) |
| Stay in less desirable job | 25.5% | 13.4% |
| Had to earn less to qualify for Medicaid | 17.0% | 9.8% |
| Worked part-time to keep total income low | 13.9% | 6.7% |
MANY HUGS CHILDREN WITH SEVERE HEMOPHILIA ARE ON A PROPHYLAXIS REGIMEN\textsuperscript{1}

Severe Hemophilia Only

<table>
<thead>
<tr>
<th></th>
<th>Children (N=109)</th>
<th>Adults (N=102)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Episodic</td>
<td>14%</td>
<td>42%</td>
</tr>
<tr>
<td>Prophylaxis</td>
<td>86%</td>
<td>58%</td>
</tr>
</tbody>
</table>

\textsuperscript{1}THE MAJORITY OF CHILDREN WITH SEVERE HEMOPHILIA ARE ON A PROPHYLAXIS REGIMEN.
PERSONS WITH SEVERE HEMOPHILIA A ON PROPHYLAXIS REGIMEN - experience significantly less bleeding episodes per year vs. episodic regimen

Annual Mean Bleeding Episodes (N=231)

- Children:
  - Mild: 4.1
  - Moderate: 9.1
  - Severe: 6.3

- Adults:
  - Mild: 5.0
  - Moderate: 8.6
  - Severe: 20.4

Annual Mean Bleeding Episodes in Severe Hemophilia patients (N=146)

- Episodic:
  - 19.8

- Prophylaxis:
  - 9.5

\( p < 0.0001 \)
In 325 patients with complete one-year clinical records, 92% reported using health services at least once:

- 28% (N=91) had emergency room visits (Mean: 0.5 visits/patient/year, Range: 0 – 8)
- 14% (N=45) were hospitalized (Mean: 5.2 days/patient/year, Range: 1 – 61)

### Episodic vs. Prophylaxis Treatment among those with Severe Hemophilia (N=205)

<table>
<thead>
<tr>
<th></th>
<th>Episodic</th>
<th>Prophylaxis</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Annual number of ER visits</strong></td>
<td>0.77</td>
<td>0.38</td>
</tr>
<tr>
<td>p</td>
<td>0.04</td>
<td>0.38</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th></th>
<th>Days</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Length of hospital stay</strong></td>
<td>12.3</td>
</tr>
<tr>
<td>p</td>
<td>0.004</td>
</tr>
</tbody>
</table>

*Only applies to patients who had a hospitalization*
QUALITY OF LIFE IN ADULTS WITH HEMOPHILIA A - Comparable to U.S. general population except for physical functioning in persons with severe hemophilia¹

QUALITY OF LIFE IN CHILDREN WITH HEMOPHILIA A - Comparable to or better than U.S. healthy population and better than that in other chronic disease populations\(^1\)

### PedsQL for Children with Hemophilia A

<table>
<thead>
<tr>
<th>Score Type</th>
<th>Mild (N=36)</th>
<th>Moderate (N=20)</th>
<th>Severe (N=109)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Physical</td>
<td>95.2</td>
<td>90.5</td>
<td>87.3</td>
</tr>
<tr>
<td>Psychosocial</td>
<td>88.4</td>
<td>82.9</td>
<td>82.8</td>
</tr>
<tr>
<td>Total</td>
<td>90.8</td>
<td>85.6</td>
<td>84.3</td>
</tr>
</tbody>
</table>

### PedsQL-Total Score for Healthy Children and Children with Other Morbidities

- Healthy: 82.7
- Card.: 79.4
- diab.: 76.6
- Obesity: 75
- GI: 72.7
- Asthma: 69.6
- Cancer: 68.7
- C.P.: 51.2

U.S. Quality of Life is indicated by a line at the 50th percentile.
### TYPES OF COSTS USED IN HEALTH ECONOMICS ANALYSIS

<table>
<thead>
<tr>
<th>Cost Category</th>
<th>Examples</th>
</tr>
</thead>
<tbody>
<tr>
<td>Direct Medical Cost</td>
<td>Laboratory test, Drugs, Hospitalization</td>
</tr>
<tr>
<td>Direct Non-Medical Cost</td>
<td>Transportation</td>
</tr>
<tr>
<td>Indirect Cost</td>
<td>Days lost from work, reduced productivity</td>
</tr>
<tr>
<td>Intangible Cost</td>
<td>Pain, suffering</td>
</tr>
</tbody>
</table>
### Table: Measuring Direct Costs for Hemophilia

<table>
<thead>
<tr>
<th>Type</th>
<th>Cost Data source(s)</th>
</tr>
</thead>
</table>
| 1. Medications                                                      | 1. Factor unit cost is estimated using average sales price (ASP) from the payment allowance limit for Medicare Part B  
  2. Average wholesale price (AWP) with 25% discounting for estimating non-nonfactor and other drug costs |
| 2. Healthcare service(s) (visits-various types)                    | Cost for CPT code from the Medicare fee schedule relative value units (RVUs)        |
| 3. Hospitalization inpatient                                       | Average cost for hospitalization (by discharge diagnosis) from Healthcare Cost and Utilization Project; adjusted by cost-to-charge ratio |
| 4. Physician inpatient                                             | Cost for CPT code from the Medicare fee schedule RVUs                               |
| 5. Emergency department                                            | MEPS, the National Ambulatory Medical Care Survey, Medicaid                          |
| 6. Outpatient procedure, laboratory tests, paid caregiver costs (SNF), Medical Devices | Cost for CPT code from the Medicare fee schedule RVUs |
# MEASURING INDIRECT COSTS FOR HEMOPHILIA

<table>
<thead>
<tr>
<th>Type</th>
<th>Cost Data source(s)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Lost work time by individuals with disease</td>
<td>The US Department of Labor Statistics “employer costs per hour worked for employee compensation”</td>
</tr>
<tr>
<td>2. Lost work time by family and friends of individual due to unpaid caregiving</td>
<td></td>
</tr>
<tr>
<td>3. Unpaid caregiving services provided by family and friends to the individual needing care</td>
<td></td>
</tr>
</tbody>
</table>
MAJORITY OF PARTICIPANTS/PARENTS ARE EMPLOYED FULL- OR PART-TIME WITH SOME UNEMPLOYMENT DUE TO HEMOPHILIA A¹

Parents of Children (N=165)

- Due to child's hemophilia: 48%
- Other reasons: 8%
- Full time: 5%

Adults (N=164)

- Due to hemophilia: 43%
- Student: 4%
- Other reasons: 19%
- Full time: 13%

Number of Patients

<table>
<thead>
<tr>
<th></th>
<th>Full-time</th>
<th>Part-time</th>
<th>Unemployed</th>
</tr>
</thead>
<tbody>
<tr>
<td>Parents of Children</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Adults</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
CHILDREN $\geq$ 5 YEARS OLD AND ADULTS - missed an average of 5.1 and 15.6 days of school/work per year respectively, due to hemophilia A$^5$

**Children $\geq$ 5 years (N=90)**

- **Mild**: 67%, 25%, 6%
- **Moderate**: 64%, 21%, 14%
- **Severe**: 60%, 14%

Mean: 5.1 days (Range: 0-7)
Median: 1 day

Excessive absenteeism defined as missing eleven or more school days. (Monahan et al. 2009)

**Adults (N=117)**

- **Mild**: 58%, 23%, 19%
- **Moderate**: 79%, 14%, 7%
- **Severe**: 62%, 12%, 26%

Mean: 15.6 days (Range: 0-270)
Median: 0 days

Average lost work days for US workers = 8.39 days (2003 National Health Interview Survey)
HIGHER FACTOR USE BY SEVERE (VS MILD/MODERATE) HEMOPHILIA A PATIENTS AND BY INHIBITOR (VS NON-INHIBITOR) PATIENTS

Annual Factor Dispensed (by Hemophilia Severity) (N=299)*

- **Children**
  - Mild (Mean: 582 IU/kg/yr)
  - Moderate (Mean: 1,089 IU/kg/yr)
  - Severe (Mean: 4,236 IU/kg/yr)

- **Adults**
  - Mild (Mean: 582 IU/kg/yr)
  - Moderate (Mean: 1,089 IU/kg/yr)
  - Severe (Mean: 4,236 IU/kg/yr)

Mean Factor VIII use (Inhibitor vs Non-inhibitor)

- **Non-inhibitors (N=299)**
  - Mean: 2,945 IU/kg/yr

- **Inhibitors (N=16)**
  - Mean: 9,046 IU/kg/yr

*Patients with current inhibitors (N=16) were excluded.
HEMOPHILIA A: HIGHER FACTOR USE BY PERSONS WITH INHIBITORS (VS NON-INHIBITOR) REFLECTED BY HIGHER ANNUALIZED FACTOR COST

Reference prices: Medicare Average Sales Price
HEMOPHILIA A WITH SEVERE HEMOPHILIA ON EPISODIC TREATMENT OR WITH INHIBITORS HAVE GREATER HEALTHCARE COSTS THAN MILD/MODERATE HEMOPHILIA OR SEVERE ON PROPHYLAXIS^4

Total Non-Factor Healthcare Costs in Hemophilia A

Costs include HTC visits, laboratory tests, emergency room (ER) visits, hospitalizations and outpatient procedures
LOST WAGES FROM WORKING PART TIME OR BEING UNEMPLOYED DUE TO HEMOPHILIA ARE MAJOR DRIVERS OF TOTAL INDIRECT COSTS OF HEMOPHILIA A

Parents of Children (N=114)

- Mild: $3,925
- Moderate: $8,498
- Severe: $8,897

Total Indirect Cost: $11,137

Adults (N=117)

- Mild: $7,716
- Moderate: $9,213
- Severe: $15,471

Total Indirect Cost: $16,485

Sensitivity analysis of annual indirect costs by varying part-time work hours from 10 hours/week to 30 hours/week


Indirect costs: Measures production loss in terms of lost earnings of the patient or caregiver. The method used here uses wages as a proxy measure of the output of work time.
TOTAL HEALTHCARE COSTS IN HEMOPHILIA A^4

Cost

$800,000
$700,000
$600,000
$500,000
$400,000
$300,000
$200,000
$100,000
$0

Mild (N=74)
Moderate (N=35)
Severe Episodic (N=67)
Severe Prophylaxis (N=123)
Inhibitor (N=16)

$3,912
$3,975
$16,185
$2,852
$42,167
$62,780
$159,761
$275,324
$13,086

Factor cost  Non-factor Healthcare cost

$100,000 $200,000 $300,000 $400,000 $500,000 $600,000 $700,000 $800,000

$0

Inhibitor (N=16)
Severe Prophylaxis (N=123)
Severe Episodic (N=67)
Moderate (N=35)
Mild (N=74)
Limitations of this Analysis

- Does not include those receiving non-HTC care
- Although representative of HTC population, over represented by persons with severe Factor VIII deficiency
- Need to weight costs by total population distribution of mild/mod/severe for calculation of total cost of illness
- A first look, but more to be done!
## Healthy People 2020 Objectives: Blood Disorders and Blood Safety Related to Hemophilia

<table>
<thead>
<tr>
<th>Goal</th>
<th>Baseline, target, improvement and potential data source</th>
</tr>
</thead>
<tbody>
<tr>
<td>Increase the number of persons with bleeding disorders who receive recommended vaccinations</td>
<td>Data: Universal Data Collection Project (UDC), CDC</td>
</tr>
<tr>
<td>Increase the number of providers who refer women with symptoms suggestive of inherited bleeding disorders for diagnosis and treatment</td>
<td>Data: American College of Obstetricians and Gynecologists (ACOG) Survey</td>
</tr>
</tbody>
</table>
| Increase the number of women with VWD who are timely and accurately diagnosed | Baseline: 28.4% of women timely and accurately diagnosed in 2008  
Target: 31.2% (10% improvement) by 2020  
Data: UDC, CDC |
| Reduce the number of persons with hemophilia who develop reduced joint mobility due to bleeding into joints | Baseline: 82.9% of persons developed reduced joint mobility due to bleeding into joints in 2008  
Target: 74.6% (10% improvement) by 2020 |
# Hemophilia-bleeding event rates (per episode)

<table>
<thead>
<tr>
<th>Title</th>
<th>Health Status Markers: “Under Control”</th>
<th>Description</th>
<th>Comments References</th>
</tr>
</thead>
</table>
| Report bleeding as an event rate per patient, in the same manner as ADEs and pADEs are reported:  
**Numerator** = # of bleeding events among POF  
**Denominator** = # of patients in POF | Proposed measure will track bleeding event rates rather than % of patients who experienced bleeding. With the exceptions of trauma-induced bleeding, bleeding events in hemophilia patients are analogous to ADEs. Therefore, bleeding as a primary outcome measure of hemophilia treatment should be tracked in the same format as ADEs and pADEs. This measure has clinical and quality improvement value. | Not from National Quality Forum (none available for hemophilia) |
ACKNOWLEDGEMENTS

- We thank our sponsors: CSL Behring, Baxter Healthcare Corporation, Bayer Foundation, Novo Nordisk for funding the HUGS Va project and Pfizer (formerly Wyeth) for funding the HUGS Va and Vb projects. Additional financial support was obtained from the Federal Hemophilia Treatment Centers/Region IX, Grifols, Red Chip, and CHOC at Home.

The Hemophilia Utilization Group Study Part-V:

- University of Southern California: Kathleen A. Johnson, PharmD, MPH, PhD (Principal Investigator), Mimi Lou, MS, Zheng-Yi Zhou, MS, Jiat Ling Poon, Xiaoli Niu, Jason Doctor, PhD, Joanne Wu, MS, Michael Goode (Programmer);
- Children’s Hospital Los Angeles, Hemostasis and Thrombosis Center: Robert Miller (Site Principal Investigator), Jennifer Hanley;
- Children's Hospital of Orange County, Hemophilia Treatment Center: Heather Huszti, PhD (Site Principal Investigator), Brandy Fitzhenry, James Fabela;
- University of Colorado Denver: Brenda Riske, MS, MBA, MPA (Site Principal Investigator), Cassie Ross, Carissa Smith, Julie Smith, Deirdre Cooper-Blacketer;
- Indiana Hemophilia & Thrombosis Center, Hemophilia Treatment Center: Amy Shapiro, MD (Site Principal Investigator), Natalie Duncan, MPH, Melissa Meyer, Brandy Trawinski, Jayme Harvey;
- UMASS Memorial Hospital, New England Hemophilia Center: Ann D. Forsberg, MA, MPH (Site Principal Investigator), Patricia Forand, RN;
- University of Texas at Houston, Gulf States Hemophilia and Thrombophilia Center: Megan M. Ullman, MA, MPH (Site Principal Investigator);
- Akron Children’s Hospital Medical Center, Ohio: Erin Cockrell, DO (Site Principal Investigator), Dawn Ali, Janice Kakish;
- Michigan State University, Center for Bleeding and Clotting Disorders: Roshni Kulkarni, MD (Site Principal Investigator), Cheryl Robins, Laura Carlson;
- Hemophilia Program, Puget Sound Blood Center, Washington: Barbara A. Konkle, MD (Site Principal Investigator), Michelleann Bonoan, Sarah Ruuska, Sarah Galdzicka, Steve Grantham. (HUGS Vb recruitment commenced October 2011, no patients represented in this data)
- The authors thank the HUGS Steering Committee: Randall G. Curtis, MBA, Shelby L. Dietrich, MD and Marion A. Koerper, MD, and Consultants Judith R. Baker, MHSA, Kathy Parish, PhD and Femida Gwadry-Sridhar, BSPhm, MS, PhD, Denise R. Globe, PhD.
REFERENCES

- **Published papers:**

- **Posters:**