The Evolving Hemophilia Managed Care and Specialty Pharmacy Environment: Recommendations for a New Health Care Ecosystem

Outcomes Tool Box

Reviewed by:

Edmund Pezalla, MD
National Medical Director for Pharmacy Policy and Strategy
Aetna, Inc.

Michael Zeglinski, RPh
Senior Vice President, Specialty Pharmacy
OptumRx®/BriovaRx®

Jointly provided by

National Hemophilia Foundation
for all bleeding disorders

Impact Education

Postgraduate Institute for Medicine

This activity is supported by independent educational grants from Baxalta US Inc., Biogen, and Novo Nordisk, Inc.
The Cost Burden of Hemophilia in Managed Care

Prevalence, Drug Utilization, and Associated Costs
Hemophilia is a Low Prevalence but High Cost Disease
Prescription Cost Vastly Outweighs Hemophilia Prevalence and Associated Utilization
Hemophilia and the Specialty Drug Trend
Pharmacy Spending on Hemophilia Products and Other Specialty Drugs is Expected to Grow
Key Drivers of Specialty Trend
Hemophilia Ranks Among the Therapeutic Classes Driving the Specialty Trend

Appropriate Hemophilia Management
Treatment Priorities, Approach, and Strategies
Treatment Priorities
Treatment Goals, Approach, and Strategies
Treatment Options for the Management of Bleeding
Treatment Options
Control and Prevention of Bleeding with Factor Replacement,
Management Challenges

Measuring Success in Hemophilia Management
Determining the Value of Care
Overall Value in Care is Based on Total Cost of Care and Care Experience Evidence
Current Sources of Data
The Need for Collaboration
Key Components of Data Collection and Analysis for Hemophilia Quality Improvement

The Comprehensive Care Sustainability Collaborative (CCSC)
Introduction
The CCSC Initiative Strives to Facilitate Payer-Provider Collaboration
Metric Development
CCSC Metric Development Process
CCSC Development of Finalized Metrics
By Reporting Data According to these Metrics, HTCs, and Payers Can Improve Outcomes and Manage Costs

Metrics for Quality Improvement
CCSC-recommended Metrics for HTCs and Payers
Patient Classification
Prescribed dose/dispensed dose/weight (± range)
Number of bleeds/time to treatment
ED visits/hospitalizations
Cost of factor
Home infusion (%)
Total cost per patient
Patient contacts

Further Information and Opportunities for Payers
Resources Are Available for Payers Seeking More Information on the CCSC
Payers and Plans Have an Opportunity to Assess Outcomes for Hemophilia through the CCSC
# The Cost Burden of Hemophilia in Managed Care

## Prevalence, Drug Utilization, and Associated Costs

**Hemophilia is a Low Prevalence but High Cost Disease**

<table>
<thead>
<tr>
<th>Condition</th>
<th>Estimated Prevalence</th>
<th>Estimated Per Patient Cost of Care ($)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Diabetes¹</td>
<td>25,800,000</td>
<td>7,900 – 14,000</td>
</tr>
<tr>
<td>COPD²</td>
<td>15,000,000</td>
<td>2,000 – 43,000</td>
</tr>
<tr>
<td>Multiple Sclerosis³,⁴</td>
<td>300,000</td>
<td>28,000 – 58,000</td>
</tr>
<tr>
<td>Hemophilia⁵</td>
<td>20,000</td>
<td>180,000 – 300,000</td>
</tr>
</tbody>
</table>

**Prescription Cost Vastly Outweighs Hemophilia Prevalence and Associated Utilization⁶**

- 0.01% Prevalence of Use
- 0.001 PMPY Prescriptions

$7,519.16 Average Cost Per Prescription
Hemophilia and the Specialty Drug Trend

Pharmacy spending on Hemophilia Products and Other Specialty Drugs is expected to grow.

Spending on Specialty Drugs Projected to Surpass Sales of Traditional Agents by 2018

<table>
<thead>
<tr>
<th>Year</th>
<th>Traditional</th>
<th>Specialty</th>
</tr>
</thead>
<tbody>
<tr>
<td>2012</td>
<td>$290</td>
<td>$665</td>
</tr>
<tr>
<td>2013</td>
<td>$348</td>
<td>$675</td>
</tr>
<tr>
<td>2014</td>
<td>$425</td>
<td>$694</td>
</tr>
<tr>
<td>2015</td>
<td>$514</td>
<td>$722</td>
</tr>
<tr>
<td>2016</td>
<td>$612</td>
<td>$751</td>
</tr>
<tr>
<td>2017</td>
<td>$722</td>
<td>$789</td>
</tr>
<tr>
<td>2018</td>
<td>$845</td>
<td>$836</td>
</tr>
</tbody>
</table>

PMPY=per member per year

Key Drivers of Specialty Trend

<table>
<thead>
<tr>
<th>High Cost Per Patient</th>
<th>Increasing Utilization</th>
</tr>
</thead>
<tbody>
<tr>
<td>Accounts for 25% of pharmaceutical spending in the US</td>
<td>Flourishing pipeline</td>
</tr>
<tr>
<td>Annual growth at 15-20%</td>
<td>New indications for existing drugs</td>
</tr>
<tr>
<td>Annual drug cost ranges from $15,000-$250,000+ per patient</td>
<td>Earlier use of biologics in treatment regimen for diseases where nonbiologic options are available</td>
</tr>
<tr>
<td>Manufacturer price increases for existing drugs</td>
<td>Episodic vs. chronic treatment</td>
</tr>
<tr>
<td>Limited generics available as products mature:</td>
<td></td>
</tr>
<tr>
<td>- First wave of non-biologic specialty drugs losing patent protection</td>
<td></td>
</tr>
<tr>
<td>- Biosimilars for biologic specialty drugs</td>
<td></td>
</tr>
</tbody>
</table>
## Hemophilia Ranks Among the Therapeutic Classes Driving the Specialty Trend

### Top Specialty Therapy Classes

**RANKED BY 2014 PMPY SPEND**

<table>
<thead>
<tr>
<th>RANK</th>
<th>THERAPY CLASS</th>
<th>PMPY SPEND</th>
<th>UTILIZATION</th>
<th>UNIT COST</th>
<th>TOTAL</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Inflammatory Conditions</td>
<td>$80.03</td>
<td>8.5%</td>
<td>15.7%</td>
<td>24.3%</td>
</tr>
<tr>
<td>2</td>
<td>Multiple Sclerosis</td>
<td>$52.36</td>
<td>3.2%</td>
<td>9.7%</td>
<td>12.9%</td>
</tr>
<tr>
<td>3</td>
<td>Oncology</td>
<td>$41.64</td>
<td>8.9%</td>
<td>11.7%</td>
<td>20.7%</td>
</tr>
<tr>
<td>4</td>
<td>Hepatitis C</td>
<td>$37.95</td>
<td>76.1%</td>
<td>666.6%</td>
<td>742.6%</td>
</tr>
<tr>
<td>5</td>
<td>HIV</td>
<td>$27.24</td>
<td>4.5%</td>
<td>10.3%</td>
<td>14.8%</td>
</tr>
<tr>
<td>6</td>
<td>Miscellaneous Specialty Conditions</td>
<td>$11.10</td>
<td>27.3%</td>
<td>8.2%</td>
<td>35.6%</td>
</tr>
<tr>
<td>7</td>
<td>Growth Deficiency</td>
<td>$9.98</td>
<td>-0.9%</td>
<td>7.5%</td>
<td>6.6%</td>
</tr>
<tr>
<td>8</td>
<td>Hemophilia</td>
<td>$5.49</td>
<td>-0.8%</td>
<td>17.6%</td>
<td>16.9%</td>
</tr>
<tr>
<td>9</td>
<td>Pulmonary Arterial Hypertension</td>
<td>$5.41</td>
<td>7.6%</td>
<td>6.2%</td>
<td>13.8%</td>
</tr>
<tr>
<td>10</td>
<td>Transplant</td>
<td>$5.13</td>
<td>0.8%</td>
<td>-3.1%</td>
<td>-2.3%</td>
</tr>
<tr>
<td><strong>TOTAL SPECIALTY</strong></td>
<td><strong>$311.11</strong></td>
<td><strong>5.8%</strong></td>
<td><strong>25.2%</strong></td>
<td><strong>30.9%</strong></td>
<td></td>
</tr>
</tbody>
</table>
APPROPRIATE HEMOPHILIA MANAGEMENT

Treatment Priorities, Approach, and Strategies

TREATMENT PRIORITIES

- Treatment priorities for persons with hemophilia
  - Prevention of bleeding
  - Immediate infusion of clotting factors if excessive bleeding does occur
  - Prevention of disability
- Advances in hemophilia care allow for a near normal life expectancy
  - Use of prophylactic (preventive) factor infusion protocols
  - Advent of longer-acting factor may lead to decreased number of infusions/week (when applicable)

TREATMENT GOALS, APPROACH, AND STRATEGIES

<table>
<thead>
<tr>
<th>Goals</th>
<th>Approach</th>
<th>Strategies</th>
</tr>
</thead>
<tbody>
<tr>
<td>Rapid and effective replacement of missing coagulation factor in order to:</td>
<td>Comprehensive hemophilia treatment center (HTC) staffed by a multidisciplinary team of experts who care for patients with bleeding disorders</td>
<td>Episodic or “on demand” factor replacement</td>
</tr>
<tr>
<td>Raise factor levels</td>
<td></td>
<td>Prophylaxis</td>
</tr>
<tr>
<td>Decrease frequency and severity of bleeding</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Prevent the complications of bleeding</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
Treatment Options for the Management of Bleeding

Treatment Options

- Replacement of missing clotting protein
  - Hemophilia A: concentrated FVIII product
  - Hemophilia B: concentrated FIX product
- Desmopressin acetate (DDAVP)/Stimate
  - Synthetic vasopressin analog used in many patients with mild hemophilia A for joint, muscle, and oro-nasal bleeding and before and after surgery
- Adjunctive therapies
  - Antifibrinolytic agents
  - Supportive measures including immobilization and rest

Control and Prevention of Bleeding with Factor Replacement

<table>
<thead>
<tr>
<th>Bleeding Episode</th>
<th>Factor Level Required (% of normal)</th>
<th>Frequency of Administration*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Minor</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Early hemarthrosis</td>
<td>30-50</td>
<td>Every 12-24 hours ± antifibrinolytic</td>
</tr>
<tr>
<td>Minor muscle or oral bleed</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Moderate</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Bleeding into muscles or oral cavity</td>
<td>50-80</td>
<td>Every 12-24 hours until resolved</td>
</tr>
<tr>
<td>Definite hemarthrosis</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Major</td>
<td></td>
<td></td>
</tr>
<tr>
<td>GI, intracranial, intra-abdominal,</td>
<td>80-100</td>
<td>Every 12-24 hours until resolved</td>
</tr>
<tr>
<td>intrathoracic, CNS, or retroperitoneal</td>
<td></td>
<td></td>
</tr>
<tr>
<td>bleeding</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Special Case Scenarios</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Patients already on prophylaxis,</td>
<td>Variable</td>
<td>Variable</td>
</tr>
<tr>
<td>patients using long-acting factor</td>
<td></td>
<td></td>
</tr>
<tr>
<td>products, etc.</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

*Recommended FVIII dosing:
Dosage in FVIII units = (Weight in kilograms) x (Factor percentage desired) x 0.5 (per product indications)
Management Challenges

- Prophylaxis\textsuperscript{12,13,14,15,16,17}
  - Identification of optimal trough level
  - Cost-benefit of targeting higher trough levels
  - Use of prophylaxis beyond pediatric patients
  - Perisurgical considerations
  - Impact of prophylaxis on CVD risk

- Formation of inhibitory antibodies\textsuperscript{18,19}
  - Genetic predisposition
  - Factor exposure during heightened immune response
    - Infections, immunizations, surgery
    - More frequent (or continuous) factor infusions in mild or moderate cases
  - Eradication of the inhibitor in severe cases
Determining the Value of Care

**Overall Value in Care is Based on Total Cost of Care and Care Experience Evidence**

\[
V = \frac{Q}{C}
\]

- Evidence-based therapies
- Minimal adverse events
- Reduced morbidity
- Improved QOL
- Avoidance of hospitalizations
- Avoidance of ED visits
- Site of service costs ↓
- Inappropriate/excessive dosing ↓
# Current Sources of Data

<table>
<thead>
<tr>
<th>Data Source</th>
<th>Description</th>
</tr>
</thead>
</table>
| All Payer Claims Databases (APCD)                   | • Most under construction; lack public payers  
• De-identified protected health information (PHI); link to provider  
• Annual updates, long claims lag; often non-specific  
• No clinical data & PBM data from carve-outs  
• Best for population level analysis (e.g., state cohort profiling) |
| Aggregated Commercial Databases                    | • Larger cross-state cohorts; less claims lag than APCDs  
• Commercial data; open for contracting  
• De-identified non-clinical data  
• Examples: BCBS, Optum, HMO Research Group |
| Health Plans                                        | • PHI included; can track specific patients  
• PBM data integrated; data limited to plan membership  
• Wide variation in availability: Humana vs. HPHC  
• No clinical data except staff model plans (e.g., Intermountain, Henry Ford Health Systems, or Kaiser Permanente) |
| Electronic Medical Records (EMRs) / Health Care Providers | • PHI, clinical, and demographic data included  
• Includes prescribed meds but no way to know if filled  
• Access & formatting variable (lab vs. imaging vs. notes)  
• No data from other providers of studied patients |
| Self-Insured Employer Groups                        | • Claims download database for large, self-funded employers  
• Health utilization and possibly work impact data included  
• Usually outsourced (e.g., Mercer, Solucia, etc.)  
• Limited by employee privacy & profile of workforce |
| Centers for Medicare and Medicaid Services (CMS)    | • De-identified  
• Very broad  
• Prescription data not integrated  
• Only available in small samples (i.e., regional data) |
| Patient-Reported Outcomes (PRO) Scheduled Data      | • Periodic Surveys  
• Notification Window  
• Email Reminders  
• Rewards  
• Challenges  
• Validated Instruments  
• Longitudinal trends |
| Real-Time Data                                      | • Event-driven Diary  
• Real Time  
• Improved Recall  
• Rewards  
• Challenges  
• Web-Only or Mobile  
• Data Verification  
• EMR Integration  
• Specialty Pharmacy (SPP) Refill Data  
• Triggered Dynamic Medical Education Content |
The Need for Collaboration

**KEY COMPONENTS OF DATA COLLECTION AND ANALYSIS FOR HEMOPHILIA QUALITY IMPROVEMENT**

**Collaboration Between Payers and Providers is Imperative**

Providers

Growing but still underutilized; will be a key feature of future payer/provider interactions

Payers

Communication between Payers and HTCs

Claims Analyses

Eventual EMR Compatibility
THE COMPREHENSIVE CARE SUSTAINABILITY COLLABORATIVE (CCSC)

Introduction

THE CCSC INITIATIVE STRIVES TO FACILITATE PAYER/PROVIDER COLLABORATION

- Ongoing quality improvement (QI) and cost management initiative
- Driven by the insights of a prominent group of stakeholders:
  - Hemophilia treatment center (HTC) directors, clinicians, and administrators
  - Payer/managed care medical and pharmacy directors from a mix of large national and regional health plans
- Developing a framework for metric-driven programs incorporating data reporting between payers and HTCs to be replicated across the United States
- Goal: facilitate cost-effective hemophilia management integrating the HTC comprehensive care model
CCSC Metric Development Process

CCSC-recommended Metrics
- Vetting and analysis by subcommittee

Intermediate Metrics
- Validation of metrics via data collected in preliminary pilots

Finalized Metrics
- For use in pilot programs for analysis and measurement

CCSC Development of Finalized Metrics
- Based on the data collection and reporting experiences presented by HTC and payer advisors participating in preliminary initiatives, a consensus was reached to revise the metrics to capture data that more accurately reflects true outcomes and costs
- Discussion of the revised metrics commenced with a model mentioned in previous CCSC recommendations, followed by eventual agreement on finalized metrics
BY REPORTING DATA ACCORDING TO THESE METRICS, HTCs, AND PAYERS CAN IMPROVE OUTCOMES AND MANAGE COSTS
**Metrics for Quality Improvement**

CCSC-recommended Metrics for HTCs and Payers

**Patient Classification**
To be reported by the HTC, as payer claims data does not provide all of the pertinent detail:
- Diagnosis (A or B)
  - Severity (mild, moderate, or severe)
  - Inhibitor status (Y or N)

**Prescribed Dose/Dispensed Dose/Weight (± Range)**
To be reported by the HTCs using an integrated pharmacy model or payers if an SPP is used for factor dispensation:
- Product
- Total units
- U/kg
- Units dispensed
- Prescribed dose/dispensed dose
  - ±10% according to MASAC guidelines; payers desire ±5%

**Number of Bleeds/Time to Treatment**
To be reported by the HTC:
- Total number of bleeds
- Type of bleed (joint or non-joint)
- Type of treatment (prophylaxis or on-demand)

**ED Visits/Hospitalizations**
To be reported by both the HTC and the payer:
- ED visit with hemophilia listed as 1st or 2nd diagnosis code (i.e., in the first two lines of the claim)
  - While payers have ED data, they do not always have the details to understand the complete details for a given patient scenario
**Cost of Factor**

To be reported by the payer:
- Total factor cost
- Total factor cost/patient
- Site of care
  - Facility (hospital/ED)
  - Ambulatory (infusion center, physician’s office, HTC)
  - Home/self

**Home Infusion (%)**

As an indicator of cost-saving home infusion, to be reported by the HTC:
- Percent of patients/families independently infusing at home
- Percent of patients/families infusing at home with nursing assistance

**Total Cost per Patient**

To be reported by the payer:
- Total cost of pharmacy claims
- All other medical claims costs
- Total cost per patient

**Patient Contacts**

As an indicator of quality care, to be reported by the HTC:
- Comprehensive care visits
- Other visits
  - Follow-ups
    - Medical provider
    - Social work
    - Nurse
    - PT
  - Patient/family education
  - Infusions
  - Offsite visits (home and school)
- Collaboration with other providers
- Telemedicine
- Case management contacts
  - Telephone
  - E-mail
  - Text
Further Information and Opportunities for Payers

Resources are Available for Payers Seeking More Information on the CCSC

CCSC White Paper
- Initial findings and recommendations from the CCSC are reported in a white paper available at: www.CCSCHemo.com
- Highlights Include:
  - Analysis of the current state of hemophilia care and the benefits of the comprehensive care model
  - Expert feedback and consensus recommendations to facilitate cost-effective hemophilia management integrating the HTC comprehensive care model
  - Information regarding competitive factor pricing and a thorough explanation of the role of 340B pricing in funding ancillary services provided at HTCs
  - Recommended HTC- and payer-reported metrics to facilitate information sharing across multiple health care stakeholders

Payers and Plans Have an Opportunity to Assess Outcomes for Hemophilia Through the CCSC

Networking with the CCSC can...
- ...assist with access to the extensive array of hemophilia-related outcomes data available from a nationwide network of HTCs
- ...provide connectivity with HTC directors and other plan/payer managers seeking more rigorous outcomes measures in care quality and cost containment in hemophilia

For more information contact: CCSC@ImpactEdu.net
REFERENCES