Track 2: Cost Optimization Strategies for Factor Replacement Therapy

This activity is supported by independent educational grants from Novo Nordisk, Inc., Baxalta, part of Shire, Biogen, and Grifols.
<table>
<thead>
<tr>
<th>Agenda</th>
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</thead>
</table>
| **Hemophilia Treatment Centers:**  
**A Cost-Effective Comprehensive Care Model** | **Michael Tarantino, MD**  
Professor of Pediatrics  
Division of Pediatric Hematology/Oncology  
University of Illinois College of Medicine  
Medical Director  
Bleeding & Clotting Disorders Institute |
| **Recommendations to Maximize Cost Outcomes** | **Joan Couden, BSN, RN**  
National Director, Bleeding Disorder Program  
Option Care |
| **Measuring Success: Tools and Resources to Document Care and Cost Outcomes of Payer and Specialty Pharmacy Hemophilia Management** | **Vanita Pindolia, PharmD, BCPS**  
Vice President, Ambulatory Clinical Pharmacy Programs  
Henry Ford Health System/Health Alliance Plan of Michigan |
| **Case Study Presentations/Faculty Idea Exchange** | Faculty Panel |
| **Audience Question and Answer Session** | All |
| **Key Takeaways and Closing Comments** | Faculty Panel |
Learning Objectives

• Describe current and evolving strategies used by managed care organizations (MCOs) and specialty pharmacy providers to facilitate high quality care for members with hemophilia

• Cite the most recent clinical recommendations for the treatment of patients with hemophilia, including prophylactic factor replacement and the role of emerging agents

• Explain hemophilia-related complications associated with inhibitor development and its significant clinical and economic consequences

• Identify processes for MCOs and specialty pharmacy providers to improve communications with HTCs

• Apply methods to enable optimal cost management of factor replacement therapy to be realized by multiple hemophilia stakeholders including MCOs and specialty pharmacy providers
Track 2: Cost Optimization Strategies for Factor Replacement Therapy

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Hemophilia Treatment Centers: A Cost-Effective Comprehensive Care Model

Michael Tarantino, MD
Professor of Pediatrics
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University of Illinois College of Medicine
Medical Director
Bleeding & Clotting Disorders Institute
Hemophilia Patients Require Health Care Across their Entire Lifespan

Age Distribution of the US Hemophilia Population

- Age of diagnosis is <2 years of age
- Life expectancy exceeds 70 years
- Older patients tend to have comorbidities (eg, CVD, HCV, and HIV)
- ~60% of hemophilia patients are insured under commercial plans (ie, both fully and self-insured plans)

Goals of Care for Patients

Health care providers are to:

• provide the best possible care available to the patient with hemophilia
• educate the patient with hemophilia
• enable the patient with hemophilia to live as normal and productive a life as possible

Patients with hemophilia have extraordinary health care needs
Comprehensive Care

Comprehensive care constitutes a multidisciplinary team of health care providers (hematologists, nurses, physical therapists, social workers, dental professionals, and more) working in collaboration with the patient and family to minimize the effects of hemophilia using prevention strategies and enlisting community support, while maximizing quality of life.
Comprehensive Care....

• ...addresses the whole person/family
• ...is collaborative
• ...is coordinated
• ...is based on education
• ...instills advocacy
• ...encourages adherence
• ...improves health-related quality of life (HRQoL)
Comprehensive Care Represents a Multidisciplinary Approach Centered on the Patient

- Hematologist
- Orthopedists
- Physical Therapists
- Laboratory Technicians
- Nurses
- Psychosocial Workers
- Dentists
Historical Perspective of Comprehensive Care

- Based on integrative public health approach\textsuperscript{1}
- Successful public health program\textsuperscript{2-4}
  - Improved health outcomes for patients
  - Reduced health care resource utilization
- Effected change in the delivery of care for patients\textsuperscript{5,6}

What is an HTC?

An HTC is a federally recognized comprehensive hemophilia treatment center that has a multidisciplinary team with expertise in the care of patients with bleeding disorders and whose staff spend a majority of their time caring for these patients.

Family-Centered Care and HTCs

- HTC multidisciplinary teams work within a framework of family-centered care
- Pivotal role of family is recognized and respected
- Families are supported in traditional roles of decision-making and care-giving
- Families’ individual styles and strengths are valued and efforts made to minimize lifestyle disruptions
- Approach requires ongoing coordination of care and communication with community based medical and social service providers and agencies
Improving Hemophilia Outcomes Through Comprehensive Care

• Comprehensive care is defined as the continuous supervision of all medical and psychological aspects affecting the patient and family

• Optimal treatment is based on:
  • Early detection and diagnosis
  • Prevention and treatment of bleeding episodes and any complications, particularly hemophilic arthropathy
  • Detection and management of inhibitors
  • Psychosocial and educational support
  • Monitor for treatment-related comorbidities
  • Coordination of care with other providers involved in management of the patient

Historical Perspective of HTCs

• First HTCs originated in the UK during the 1940s\textsuperscript{1}
• Other countries soon followed – France, US, Australia, Sweden, Japan, Italy, Israel
• Have become mainstay for treatment of patients with hemophilia\textsuperscript{2}
• Developing countries with HTCs report improved survival\textsuperscript{3}
• Recommended as the model of care by World Federation of Hemophilia (WFH), World Health Organization (WHO)\textsuperscript{4}

HTCs Serve a Patient Population with Severe Bleeding Disorders and Complex Comorbidities

<table>
<thead>
<tr>
<th>Characteristics</th>
<th>HTC (%)</th>
<th>Non-HTC (%)</th>
<th>P</th>
</tr>
</thead>
<tbody>
<tr>
<td>Severity</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Mild</td>
<td>21.8</td>
<td>52.8</td>
<td>&lt;.001</td>
</tr>
<tr>
<td>Moderate</td>
<td>24.2</td>
<td>26.7</td>
<td></td>
</tr>
<tr>
<td>Severe</td>
<td>54.0</td>
<td>20.5</td>
<td></td>
</tr>
<tr>
<td>Inhibitors</td>
<td>6.0</td>
<td>2.3</td>
<td>&lt;.001</td>
</tr>
<tr>
<td>Liver disease</td>
<td>2.3</td>
<td>0.7</td>
<td>.002</td>
</tr>
<tr>
<td>HIV infection</td>
<td>31.1</td>
<td>17.1</td>
<td>&lt;.001</td>
</tr>
<tr>
<td>AIDS</td>
<td>8.2</td>
<td>5.9</td>
<td>.02</td>
</tr>
</tbody>
</table>

Benefits of Comprehensive Care: Initial Findings

Background/Methods

• Federal funding established 1975 (PL 9463)
• 11/22 comprehensive HTCs reported
• Standardized data collection form used

Results

• 2,112 patients seen in 11 HTCs increased to 4,742 at end of study
• 514 patients on self-infusion increased to 2,001
• 36.0% of patients unemployed prior to HTC care decreased to 12.8% four years later
• Days lost from work or school decreased from 14.5/y (9.4 inpatient) to 4.3/y (1.8 inpatient)
• Hospital admissions decreased from 1.9/y to 0.26/y
• Insurance coverage increased from 74% to 93%
• Annual per patient cost of care decreased from $15,800 to $5,932

Benefits of Care Delivered Through an HTC: Mortality and Hospitalization

For Patients Receiving Care via an HTC: Mortality Rate Decreases by 40% and Hospitalization Rate Decreases by 40%

Relative Mortality\(^1\)

Relative Number of Hospitalizations\(^2\)

HTC=hemophilia treatment center.

HTCs Across the Nation are Organized into a Regional Network

Mountain States
11 HTCs
2600 (8%)
University of Colorado - Denver

Western
14 HTCs
4072 (13%)
Children’s Hospital - Orange City

Northern States
16 HTCs
3747 (12%)
Great Lakes Hemophilia

Great Lakes
21 HTCs
5557 (17%)
Hemophilia of Michigan

New England
22 HTCs
4513 (14%)
University of Massachusetts

Mid-Atlantic
17 HTCs
3507 (11%)
Children’s Hospital of Philadelphia

Great Plains
15 HTCs
3518 (11%)
University of Texas Gulf States Hemophilia & Thrombophilia Center (GSHTC)

Southeast
24 HTCs
4503 (14%)
University of North Carolina - Chapel Hill
Additional Functions of the HTC

- Other major functions include
  - Establish and maintain regional and national registries of patients with bleeding disorders to document hemophilia treatment and its outcomes
  - Support and conduct basic and clinical research, particularly through participation in collaborative trials
  - Prepare treatment protocols or guidelines
  - Conduct pharmacosurveillance
  - Cooperate with the competent authorities in designing health care policies for hemophilia patients

HTC=hemophilia treatment center.

HTCs Help Control the Cost of Care

• Many unbilled services provided as part of annual visit
• Expert care limits complications
  • Multidisciplinary team
• Minimize emergency department (ED) visits
• Promote independence
• Complete medical history readily available
  • Optimal decision making
• Relationships with expert subspecialists
Value of Hemophilia Treatment Centers Across Medical and Pharmacy Benefits

Provide comprehensive care including physical, emotional, psychological, educational, and financial support

- Focus on teaching self-administration to promote independence typically by age 8
- Members show improved health with reduced costs by lowering rates of unemployment, emergency room visits, hospital stays, and illness-related time off from work and school\(^2\)
- Most members visit HTCs at least annually for comprehensive medical evaluation
- Medication can be dispensed by a pharmacy within the HTC or working in conjunction with the HTC

About **70%–80%** of people with hemophilia are under the care of an HTC\(^1\)

**HTC Care Providers**
- Hematologists
- Pediatricians
- Dentists
- Physical therapists
- Orthopedists
- Nurses
- Social Workers
- Nutritionists

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Summary

• The HTC model of care is aligned with payer approaches to managing low-prevalence, high-cost conditions

• “Carve-out” disease management of a rare disease can be highly effective, as demonstrated by the clinical successes of HTCs, but only if the aforementioned precepts are followed

• Care management is highly valuable; however, for practical purposes in managed care, the engagement mechanism must be reliable and information must be shared and actionable
  • This highlights the importance of clear quality and efficiency metrics, transparency, and consistent information sharing/data reporting
Track 2: Cost Optimization Strategies for Factor Replacement Therapy

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Recommendations to Maximize Cost Outcomes

Joan Couden, BSN, RN
National Director, Bleeding Disorder Program
Option Care
The Total Cost of Hemophilia Care is High

• ~90% of the costs of hemophilia care are attributed to the cost of factor product\textsuperscript{1,2}
  • Annual cost of factor therapy is ~$140,000/patient

• Many variables impact the total cost of care
  • Attention often focused primarily on the factor \textbf{price}, but not on key variables that impact the actual total “\textit{cost of care}”
  • Lower unit pricing from a provider lacking clinical expertise in managing these variables, and/or adequate assay inventory, can result in a significant increase in total cost of care (pharmacy/medical benefit)

Variables Impacting the Total Cost of Hemophilia Care

- Disease severity
- Bleed location/severity
- Frequency of infusions
- Target joint/inhibitors
- Promptness of treatment
- Compliance to treatment
- Coexisting medical conditions
- Use of appropriate preventive measures/in-home safety
- Use of adjunctive therapies
- HTC utilization/compliance
- Method of infusion
- Site of care (Home, HHA, HTC)
- Patient weight/activity level
- Stress/injury/surgery
- Factor dose prescribed vs dose dispensed
Specialty Pharmacy Providers Provide an Array of Services for Patients with Bleeding Disorders

Services Designed to Improve the Overall Quality of Care and Manage Disease-Related Costs

**SPP Services**
- Home delivery of factor replacement
- Assay management
- Initial clinical assessment
- Ongoing clinical oversight
- Patient education/on-demand consultation
- Treatment logs

**Outcomes**
- Improved access to care/home therapy
- Reduced variance in dispensed dose
- Improved adherence
- Reduced bleeding complications
- Reduced ER visits/hospitalizations/hospital LOS

LOS=length of stay
Consequences of a Lack of Access to Factor

• Causes delayed treatment

• Increases morbidity and mortality

• Increased morbidity can result in increased cost
  • Available guidelines recommend immediate treatment of bleeding episodes and hence promote the utilization of home-based care whenever possible
  • A lack of immediate treatment may result in further complications and increased factor utilization to resolve bleeding episodes, thereby increasing costs

Providing Clotting Concentrate in the Home Setting

• MASAC Recommendation 188:
  • Specialty pharmacy providers should be:
    • Knowledgeable about factor and ancillary supplies
    • Keep factor and supplies on hand
      • Not acceptable to merely dispense a box of factor
      • Need to provide supplies that will facilitate ease of administration
    • Be able to fill orders within 48 hours
      • SPPs and HTCs collaborate to work with families to minimize emergency dosing or need for crisis dispensing

MASAC=Medical and Scientific Advisory Committee of the National Hemophilia Foundation
Who Keeps Factor At Home?

• Severe patients on prophylaxis often have factor levels that are equivalent to, or even much lower than, those of patients with mild hemophilia and can experience breakthrough bleeding
  • Emergency doses should be kept at home in addition to the regular prophylactic doses for moderate-to-severe patients on prophylaxis

• Mild/moderate patients
  • Those who do not have local access to clotting factor/live a great distance from factor supply
  • Moderate patients who experience easily precipitated or spontaneous bleeds

• Moderate-to-severe patients
  • Those treating on demand
Patient: John Doe
Infuse 1800 to 2000 units prn
or
Infuse ± 2000 units prn

*Each Rx has potential for large variances built in*

R. Doktor, MD

Assay sizes vary from lot to lot

Factor is manufactured in a range of unit or assay sizes

- Low range
  - ~250 IU

- Mid range
  - ~500 IU

- High range
  - ~1000+ IU
Factor Rx: Assay Management

- **Assay Management**: Process of filling the prescription as closely to the prescribed target dose (bigger is better/more inventory = more assays)

- **Cost to the payer**: Depends on the total # of units actually dispensed

- **MASAC #188**: Recommends dispensing within ± 5-10% of prescribed target dose, barring extenuating circumstances

---

**John Doe**

Infuse 1800 to 2000 units prn

or

Infuse + 2000 units prn

- 800 + 1000 vial = 1800 IU
- 875 + 1100 vial = 1975 IU
- 1000 x 2 vials = 2000 IU
- 1100 x 2 vials = 2200 IU

**Available Assays:**

- 800 IU
- 875 IU
- 1000 IU
- 1100 IU
What is Disease Management?

• Multidisciplinary, continuum-based approach to healthcare delivery for a specific condition
• Supports the physician/patient relationship and plan of care
• Emphasizes prevention complications
• Uses cost-effective guidelines and patient-empowerment strategies, such as education and facilitation of self-management
• Includes continuous monitoring of outcomes with goal of improving overall health

Offerings of a High-Touch Specialty Pharmacy Program

• Patient-risk stratification
• Nurses specialized in bleeding disorders
• Initial and follow-up nursing assessments and clinical interventions
• Patient/caregiver education regarding medications and disease management
• Pharmacist assessment
• Assay management
• Dose on hand management
• Pre- and post-operative education and support
• Clinical/pharmacy staff on-call 24/7
• Clinical data collection and reporting (health plan and physician)
• Dedicated patient care coordinators
• Immune tolerance program
• Overnight or even same-day delivery
• Emergency disaster preparation
• Patient educational materials and handbooks
• Bleeding disorders newsletters
• New patient pharmacy starter kit
• Infusion logs
• Collaboration with all members of the health care team
Delivery of High-Touch Specialty Pharmacy Services Requires a Team Effort

- Coordination between all departments to provide the patient with the best care
- Patient receives confidential deliveries at the location of their choice
- Refills, regimen compliance, risk assessment, and inventory management
- Access to licensed clinicians 24 hours/day
- Targeted education, adherence coaching, and side effect management to improve clinical outcomes
- Coordination and collaboration with HTC/prescriber, as necessary, for high risk or non-compliant patients
Poor Compliance With Therapy is a Common Challenge in Hemophilia

- Similar to other chronic conditions, compliance to hemophilia therapy is a challenge\(^1,2\)
- HTCs report \(~50\%\) of their patients are not adherent to their prescribed therapy regimen\(^3\)

![Self-reported Compliance to Hemophilia Therapy\(^4\) (n=52)]

<table>
<thead>
<tr>
<th>Compliance (%)</th>
<th>Excellent (&gt;76% of infusions)</th>
<th>Good (51-75% of infusions)</th>
<th>Fair (26-50% of infusions)</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>58.8%</td>
<td>26.5%</td>
<td>14.7%</td>
</tr>
</tbody>
</table>

3. Informal survey of selected HTC nurses.
Common Compliance Issues Reported with Hemophilia Treatment

- Inappropriate dose: under- or over-dosing of factor
- Delay of >3 hours before treating
- Not using RICE
- Not using protective devices
- Inappropriate timing of daily prophylaxis dosing
- Not maintaining daily immune tolerance regimen
- Mismanagement of factor and supply inventory
- Venous access difficulties
- Failure to keep scheduled HTC visits
- Failure to complete infusion logs/track bleeds
- Failure to wear emergency medical identification
- Psychosocial issues
Bleed Prevention Can Minimize Costs

Strategies to minimize continuing bleeds:

• **Managing adherence**
  • Appropriate dosing
  • Use of RICE (rest, ice, compression, elevation)
  • Prompt treatment

• **Use of therapeutic devices**
  • Reduce continuing bleeds
  • Reduce probability of target joint development

• **Hypothetical economic impact of bleed prevention**
  • 20 additional bleeds requiring 40 to 60 additional treatments at an average dose of 1000-1500 units/infusion can result in costs upwards of $60,000
  • However, prophylaxis regimens also increase overall factor utilization and related costs
Avoiding Unnecessary ED Visits Can Reduce Costs

• **Common reasons for ED visits**
  - No factor on hand
  - No supplies
  - Venous access difficulties
  - Lack of in-home nursing availability to evaluate/infuse

• **Problems**
  - Treatment delays
  - Inappropriate evaluation results in unnecessary costs
  - Lack of availability of prescribed factor product
  - Inexperienced healthcare providers

• **ED visits increase costs**
Steps to Avoid Unnecessary ED Visits

• Monitor factor inventory and infusion supplies in the home
• Patient/family education and support
• Home infusion training
• Home nursing services
• 24/7 availability
  • Emergency delivery of product
• Travel packs
• Emergency medical identification
Summary

• The cost of factor is a significant driver of the overall cost of hemophilia care; multiple variables impact factor use.

• Hemophilia disease management enhances appropriate utilization of factor and can improve treatment outcomes while minimizing costly complications.

• SPPs offer several interventions to facilitate care quality and cost containment which may include improved access to care, treatment adherence, and assay management.
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Measuring Success: Tools and Resources to Document Care and Cost Outcomes of Payer and Specialty Pharmacy Hemophilia Management

Vanita Pindolia, PharmD, BCPS
Vice President, Ambulatory Clinical Pharmacy Programs
Henry Ford Health System/Health Alliance Plan of Michigan
Hemophilia Opportunities for Health Plans

• Capitalizing on the capabilities of, and enhancing relationships with, HTCs, SPPs, and NHF
  • Collaboration to achieve higher quality and more cost-effective care

• Encouraging care that is consistent with best clinical practices
  • Apply evidence-based guidelines, such as MASAC Quality of Care Guidelines

• Better understanding of needs and coordination of care between HTCs, community hematologists, SPPs, and payers
Hemophilia Issues for Health Plans

• Potential impact of cost-sharing on patients’ therapeutic adherence
  • Cost-sharing is growing in response to overall healthcare premium pressures

• Examining the potential of investment in care today to achieve improved long-term clinical outcomes and cost savings
  • Impact of different levels of inhibitors on approaches to short-term care that can yield long-term cost savings
Documenting Hemophilia Care and Cost Outcomes Represents Both an Opportunity and an Issue for Plans

- Access to a variety of data is critical for assessing care trends and tailoring quality improvement interventions
  - Financial trend data related to specialty drug utilization
  - Assay variance
  - Clinical trend data
  - Patient clinical outcomes
- Specific components of this data are only immediately accessible by certain providers
  - Hence, collaboration with HTCs and SPPs is instrumental in this process
Financial Trend Reporting is Important to Assess the Economic Impact of Drug Utilization

- Monitor Drug Utilization for Total Cost of Therapies
- Monitor Plan Cost by Age Group
  Increasing weight and activity level impact on cost
- Monitor Disease Severity Mix and Impact on Cost

**Top 10 Hemophilia Products**

![Table showing the top 10 hemophilia products with their Q4 total claims and cost.]

**Financial Review: Plan Cost/Patient by Age Q3 & Q4**

<table>
<thead>
<tr>
<th>Age Range</th>
<th>Q3 Billed Claim Amount/Patient</th>
<th>Q4 Billed Claim Amount/Patient</th>
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<tbody>
<tr>
<td>(1-5)</td>
<td>$35,185</td>
<td>$41,233</td>
</tr>
<tr>
<td>(6-10)</td>
<td>$59,125</td>
<td>$51,329</td>
</tr>
<tr>
<td>(11-17)</td>
<td>$114,162</td>
<td>$119,814</td>
</tr>
<tr>
<td>(18-30)</td>
<td>$62,980</td>
<td>$66,559</td>
</tr>
<tr>
<td>(31-40)</td>
<td>$71,550</td>
<td>$83,814</td>
</tr>
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**Diagnosis of Patient - Q3 & Q4**

- Total Plan Cost for Factor VIII deficiency

![Pie chart showing the percent of billed claims by severity level.]

- Mild: $18,831
- Moderate: $376,024
- Severe: $4,287,920

Specialty Pharmacy Provider Sample Analyses. 2015.
Assay Variance Reporting to Track Volume of Factor Utilized

### Assay Variance Q4

<table>
<thead>
<tr>
<th>Total Prescribed Dose</th>
<th>Total Number of Doses</th>
<th>Total Dispensed Dose</th>
<th>Total Units Prescribed</th>
<th>Total Units Dispensed</th>
<th>Rx Dispensed Less Prescribed Dose</th>
<th>Total Average Assay Variance</th>
</tr>
</thead>
<tbody>
<tr>
<td>700,078</td>
<td>19,882</td>
<td>701,556</td>
<td>6,273,077</td>
<td>6,306,974</td>
<td>33,897</td>
<td>101.04</td>
</tr>
</tbody>
</table>

**Monitoring of Assay Variances**

**Aggregate Patient Assay Variances**

Specialty Pharmacy Provider Sample Analyses. 2015.
Clinical Trend Reporting Provides Details on Active Bleeding and Treatment Approach

Monitor Increase or Decrease of “Active” Bleeds

Monitor Changes/Trends in Treatment Approach

Targeted Patient Education Regarding Prior Activity
Clinical Trend and Patient Outcomes Reporting Offer Insights on Bleeding Event Risk and Resource Utilization

<table>
<thead>
<tr>
<th>Clinical Trend Reporting</th>
<th>Patient Outcomes Reporting</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Monitor bleed site for potential “target joints”</td>
<td>• Monitor increases or decreases in hospital and ER visits</td>
</tr>
<tr>
<td>• Monitor patient movement amongst risk levels</td>
<td>• Monitor patient productivity</td>
</tr>
<tr>
<td>• Monitor time required for resolution of bleed and site</td>
<td>• Monitor reasons for hospital and ER visits</td>
</tr>
</tbody>
</table>

Specialty Pharmacy Provider Sample Analyses. 2015.
Clinical Outcomes Monitoring/Reporting Provides an Overview of Ongoing Care

Case Study – Risk Management: Q3 & Q4

8 y/o male
Dx: IV deficient
Severity: Moderate
Weight: 30.5kg
No hx of inhibitor
co-infection: no
NKA

Clinical Risk Management:
Initial clinical assessment patient history collected
- Head trauma in MVA-2005, requiring craniotomy
- Immunizations: UTD
- Surgeries: Rt femur & craniotomy
- PCP, Orthopedist, Hematologist into obtained
- Homecare assessment: Pt in Medical Foster Home
- Psychosocial: 6 siblings, in need of medical follow-up Education
- History of target joints:
  - R. knee & L. ankle.
- Port placement

Careplan & Management:
- Port placement surgery (12/23)
- Mobility. Risk of infection/CVA
- Poor dentition-decay and gingivitis, dental procedures completed
- 10/17 — Risk Level 3: Patient experiencing ankle joint bleed. Patient to administer factor and also recommended that family apply ice and elevate. The family will watch for increased swelling to the ankle joint. Rx received for shipment of Benefix, amicar and supplies
- 10/17 — Physician Intervention: Call from Dr. discussed the referral for this patient, he is going to be seen by
There is a hx of a motor vehicle accident in 2005 with him sustaining some sort of injury, possibly to the RT Knee per mom she states. His target is his RT KNEE which does appear larger than the Left. Mom apparently has not followed up for over a year, which concerns the HTC. He is scheduled for a PEDIATRIC ORTHO appt on Monday 10/20. Mother contacted today and also reminded of her appt for ortho as well.
- 10/20 — RPh & Nurse Intervention-Careplan — established for monitoring of bleed resolution
- 11/11 — Risk Level 1: Patient follow-up for risk- Patient is doing well and has medication on hand. Swelling at the ankle joint has decreased-bleed resolution.
- 12/8 — Risk Level 3: Patient going in for Port placement surgery on 12/15. He is infused at school by a nursing agency, due to the difficulty the nurse is having getting a vein, he will be having a Port placed. Send 10 doses of Benefix, saline and supplies
HTCs are Accountable for Data Reporting at a National Level

• Organized according to the American Thrombosis and Hemostasis Network (ATHN) and Regional Core Centers

• Health Resources and Services Administration (HRSA)
  • Maternal and Child Health Bureau (MCHB)
  • Genetic Services Branch

• Centers for Disease Control and Prevention (CDC)
  • National Center on Birth Defects and Developmental Disabilities
  • Division of Blood Disorders
Requirements for National Data Collection

• Reporting requirements are detailed in handouts, does not include progress reports and activities

• National statistics are important to demonstrate the following:
  • Population served
  • Need
  • Impact of initiatives (eg, women with bleeding disorders, inhibitors)

• Reporting justifies continued allocation of funds

• Federal data reporting requirements often differ from data typically desired by payers
Key Components of Data Collection and Analysis for Hemophilia Quality Improvement

Collaboration Between Payers and Providers is Imperative

Growing but still underutilized, this electronic connectivity component will be a key feature of future payer/provider interactions.
The CCSC Initiative Strives to Facilitate this 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 pilot 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 (complete)

- **Intermediate Metrics**
  - Validation of metrics via data collected in mini-pilots (complete)

- **Finalized Metrics**
  - For use in pilot programs for analysis and measurement (next phase)
Finalized Metrics

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)
Finalized Metrics

Prescribed dose/dispensed dose/weight
(±range)

To be reported by the HTC using an integrated pharmacy model, or payer 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%

*Crucial for payers*
Finalized Metrics

*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)
Finalized Metrics

*ED visits/hospitalizations*

To be reported by both the HTC and the payer:

- ED visit with hemophilia listed as 1° or 2° diagnosis code (ie, in the first two lines of the claim)
  - While payers have ED data, they do not always have the information to understand the complete details for a given patient scenario
Finalized Metrics

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
Finalized Metrics

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
Finalized Metrics

*Total cost per patient*

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

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
Summary

• Data collection and reporting on the part of both payers and HTCs can be used to identify best practices and areas for care process improvements

• For those HTCs and individual providers routing factor dispensation through SPPs, these organizations likewise play an important role in data reporting

• Transparency and communication are key to this collaborative process, which may be facilitated by quality metrics and pilot programs recently developed as part of the CCSC initiative
Track 2: Cost Optimization Strategies for Factor Replacement Therapy

This activity is supported by independent educational grants from Novo Nordisk, Inc., Baxalta, part of Shire, Biogen, and Grifols.
Case Study

Douglas McKell, MS, MSc

Faculty Panel
Case 1: Gary
A 59-year-old Male with Severe Hemophilia A
Background

• Gary has severe hemophilia A with a long-standing inhibitor, and comorbid type 2 diabetes

• He has historically been managed by both an HTC and a private practice hematologist
  • Gary sees his hematologist more frequently due to his distance from the HTC

• Gary has been experiencing at least 2 bleeds per month in his knees
  • He had surgery 5 years prior in right knee

• He has worsening range of motion in both knees and is becoming less active as a result
  • He has experienced subsequent weight-gain and worsening glycemic control
Clinical Management Approach

• Gary sought care specifically from the HTC for more aggressive care due to his worsening knee function and its consequences

• During a scheduled visit with the hematologist and physical therapist at the center, Gary was consulted regarding a management approach including the following:
  • 3x weekly aPCC
  • 6 weeks of 3x weekly PT with the HTC physical therapist

• This strategy ultimately prevented bleeding episodes and has improved his mobility, allowing the patient to continue exercising and begin to shed excess weight
Faculty Discussion

• Clinical
  • Describe clinical scenarios in which bypassing agents would be used to manage a patient with an inhibitor rather than attempting ITI.
  • What are the advantages and drawbacks of such an approach in terms of bleed prevention, adverse events, etc?

• Managed Care
  • From a payer perspective, are potential cost-offsets—such as those related to obesity and comorbid conditions directly affected by mobility—considered in the payment of individual claims?
  • How else are the relatively high costs of specialty drug products reconciled when looking at members’ claims?
  • How do payers take potential cost-offsets into account when formulating clinical policy and precertification criteria?
Case 2: Darren
A 22-year-old Male with Severe Hemophilia A
Background

- Darren was diagnosed with severe hemophilia A in infancy and has been relatively well-managed his whole life.
- He works for his uncle’s small business with no paid vacation time and is typically averse to missing work days for medical reasons.
- Darren has worsening arthropathy in right elbow that has become burdensome in his daily activities.
HTC and SPP Coordination

• During a follow-up for his elbow after Darren’s annual comprehensive care visit, surgery is planned

• He expresses concerns about missing work
  • Rather than hospitalizing him for post-surgical prophylaxis, the care team at the HTC makes arrangements to coordinate in-home prophylaxis through an SPP

• The SPP provides Darren with a continuous infusion pump and educates him on how to change the bags, allowing him to continue working in his uncle’s office answering the phone and replying to e-mails
Faculty Discussion

• Clinical
  • From a clinical perspective, what factors determine an appropriate perisurgical prophylaxis regimen?
  • From a provider standpoint, what kind of documentation is necessary to prescribe on-demand factor for home use?

• Managed Care
  • Compare total costs as well as individual costs associated with in-home infusion of prophylactic factor coordinated through an SPP versus the hospital inpatient setting. What factors are taken into consideration in the approval of such claims?
    • In what scenarios would hospital stay for post-surgical prophylaxis be absolutely necessary for a patient, thereby negating the option of prophylaxis in the home setting?
    • How do payers reconcile such site of care issues? What role do patient characteristics, treatment-specific details, and benefit design play in such decisions?
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