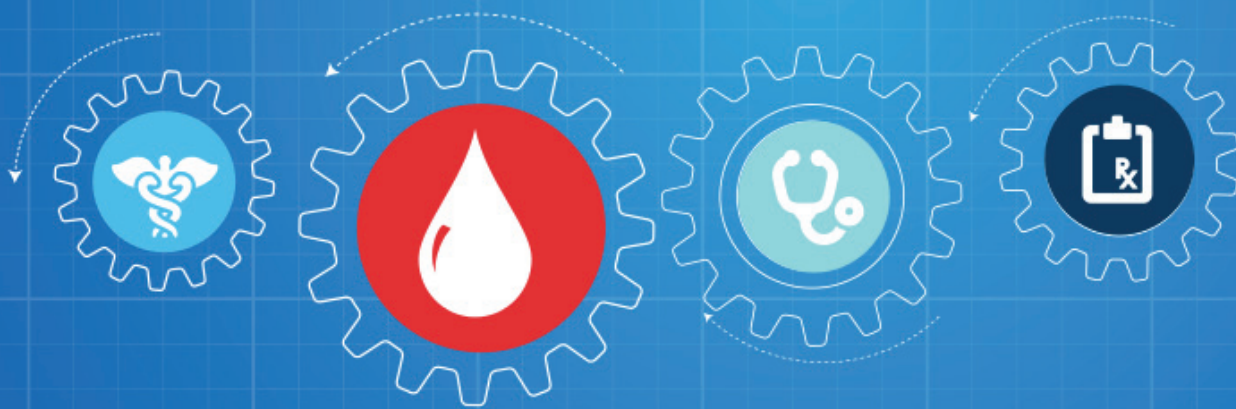


The Evolving Hemophilia Managed Care and Specialty Pharmacy Environment:

Recommendations for a New Health Care Ecosystem



Outcomes Tool Box

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NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders



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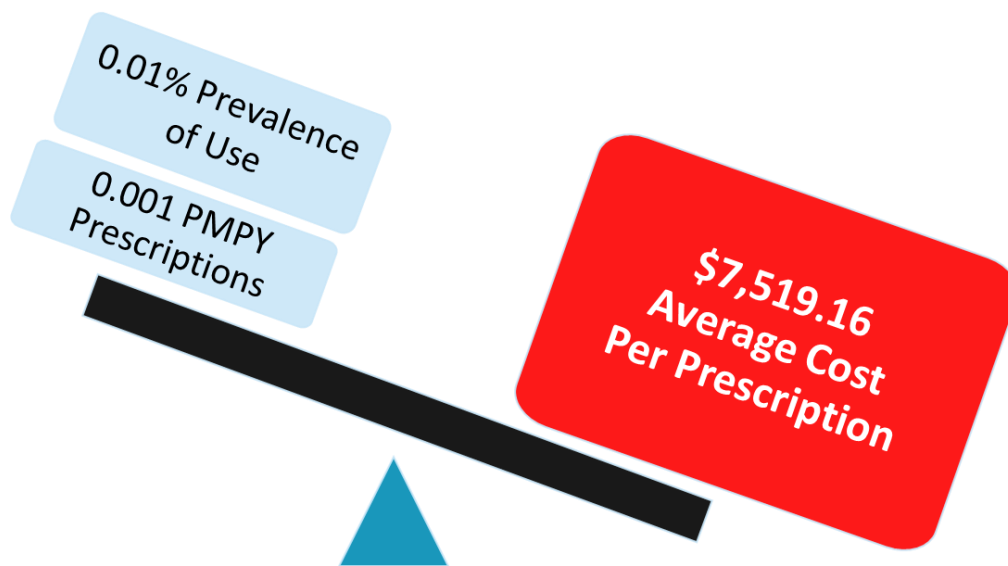
THE COST BURDEN OF HEMOPHILIA IN MANAGED CARE

Prevalence, Drug Utilization, and Associated Costs

HEMOPHILIA IS A LOW PREVALENCE BUT HIGH COST DISEASE

Condition	Estimated Prevalence	Estimated Per Patient Cost of Care (\$)
Diabetes ¹	25,800,000	7,900 – 14,000
COPD ²	15,000,000	2,000 – 43,000
Multiple Sclerosis ^{3,4}	300,000	28,000 – 58,000
Hemophilia ⁵	20,000	180,000 – 300,000

PRESCRIPTION COST VASTLY OUTWEIGHS HEMOPHILIA PREVALENCE AND ASSOCIATED UTILIZATION⁶

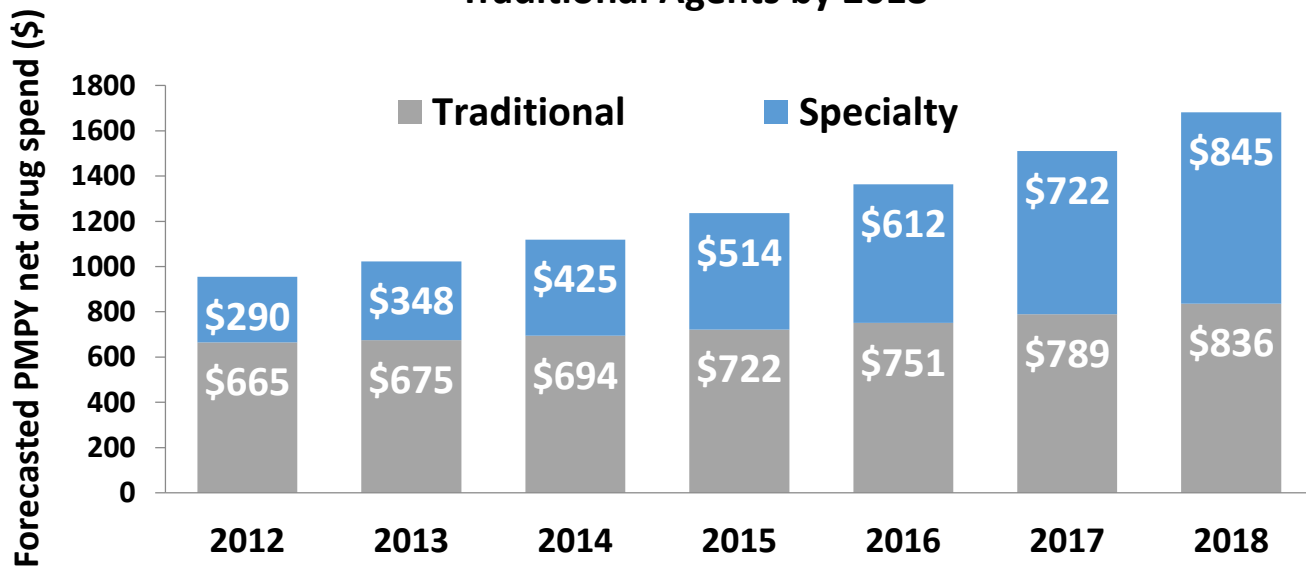




Hemophilia and the Specialty Drug Trend

PHARMACY SPENDING ON HEMOPHILIA PRODUCTS AND OTHER SPECIALTY DRUGS IS EXPECTED TO GROW⁷

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



PMPY=per member per year

KEY DRIVERS OF SPECIALTY TREND

High Cost Per Patient	Increasing Utilization
Accounts for 25% of pharmaceutical spending in the US	Flourishing pipeline
Annual growth at 15-20%	New indications for existing drugs
Annual drug cost ranges from \$15,000-\$250,000+ per patient	Earlier use of biologics in treatment regimen for diseases where nonbiologic options are available
Manufacturer price increases for existing drugs	Episodic vs. chronic treatment
Limited generics available as products mature: <ul style="list-style-type: none"> ▪ First wave of non-biologic specialty drugs losing patent protection ▪ Biosimilars for biologic specialty drugs 	



HEMOPHILIA RANKS AMONG THE THERAPEUTIC CLASSES DRIVING THE SPECIALTY TREND⁸

TOP SPECIALTY THERAPY CLASSES

RANKED BY 2014 PMPY SPEND

RANK	THERAPY CLASS	PMPY SPEND	TREND		
			UTILIZATION	UNIT COST	TOTAL
1	Inflammatory Conditions	\$80.03	8.5%	15.7%	24.3 %
2	Multiple Sclerosis	\$52.36	3.2%	9.7%	12 .9%
3	Oncology	\$41.64	8.9%	11.7%	20.7 %
4	Hepatitis C	\$37.95	76.1 %	666.6%	742.6 %
5	HIV	\$27.24	4.5%	10.3%	14.8 %
6	Miscellaneous Specialty Conditions	\$11.10	27.3%	8.2%	35.6 %
7	Growth Deficiency	\$9.98	-0.9%	7.5%	6.6 %
8	Hemophilia	\$5.49	-0.8%	17.6%	16.9 %
9	Pulmonary Arterial Hypertension	\$5.41	7.6%	6.2%	13.8 %
10	Transplant	\$5.13	0.8%	-3.1%	-2.3 %
TOTAL SPECIALTY		\$311.11	5.8%	25.2%	30.9%



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 STRATEGIES⁹

Goals	Approach	Strategies
<ul style="list-style-type: none">• Rapid and effective replacement of missing coagulation factor in order to:<ul style="list-style-type: none">○ Raise factor levels○ Decrease frequency and severity of bleeding○ Prevent the complications of bleeding	<ul style="list-style-type: none">• Comprehensive hemophilia treatment center (HTC) staffed by a multidisciplinary team of experts who care for patients with bleeding disorders	<ul style="list-style-type: none">• Episodic or “on demand” factor replacement• Prophylaxis



Treatment Options for the Management of Bleeding

TREATMENT OPTIONS¹⁰

- 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^{10,11}

Bleeding Episode	Factor Level Required (% of normal)	Frequency of Administration*
Minor <ul style="list-style-type: none"> • Early hemarthrosis • Minor muscle or oral bleed 	30-50	Every 12-24 hours ± antifibrinolytic
Moderate <ul style="list-style-type: none"> • Bleeding into muscles or oral cavity • Definite hemarthrosis 	50-80	Every 12-24 hours until resolved
Major <ul style="list-style-type: none"> • GI, intracranial, intra-abdominal, intrathoracic, CNS, or retroperitoneal bleeding 	80-100	Every 12-24 hours until resolved
Special Case Scenarios <ul style="list-style-type: none"> • Patients already on prophylaxis, patients using long-acting factor products, etc. 	Variable	Variable

*Recommended FVIII dosing:

Dosage in FVIII units = (Weight in kilograms) x (Factor percentage desired) x 0.5 (per product indications)



Management Challenges

- Prophylaxis^{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^{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



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

$$V = \frac{Q}{C}$$

- 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

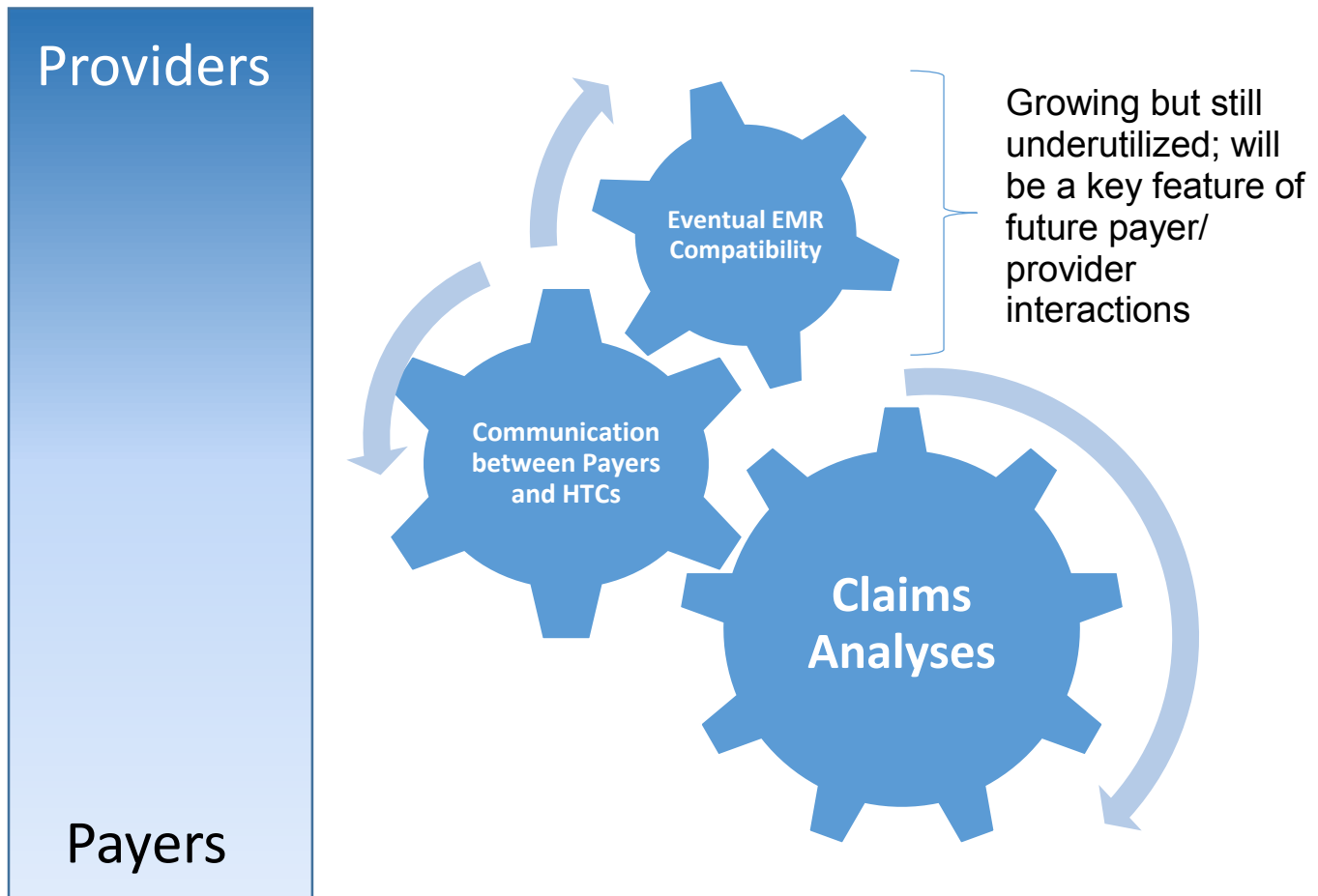
Data Source	
All Payer Claims Databases (APCD)	<ul style="list-style-type: none"> • Most under construction; lack public payers • De-identified protected health information (PHI); link to provider • Annual updates, long claims lag; often non-specific • No clinical data & PBM data from carve-outs • Best for population level analysis (e.g., state cohort profiling)
Aggregated Commercial Databases	<ul style="list-style-type: none"> • 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	<ul style="list-style-type: none"> • 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	<ul style="list-style-type: none"> • 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	<ul style="list-style-type: none"> • 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)	<ul style="list-style-type: none"> • De-identified • Very broad • Prescription data not integrated • Only available in small samples (i.e., regional data)
Patient-Reported Outcomes (PRO) Scheduled Data	<ul style="list-style-type: none"> • Periodic Surveys <ul style="list-style-type: none"> • Notification Window • Email Reminders • Rewards • Challenges • Validated Instruments <ul style="list-style-type: none"> • Longitudinal trends
Real-Time Data	<ul style="list-style-type: none"> • Event-driven Diary <ul style="list-style-type: none"> • Real Time • Improved Recall • Rewards • Challenges • Web-Only or Mobile • Data Verification <ul style="list-style-type: none"> • 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





THE COMPREHENSIVE CARE SUSTAINABILITY COLLABORATIVE (CCSC)

Introduction

THE CCSC INITIATIVE STRIVES TO FACILITATE PAYER/PROVIDER COLLABORATION

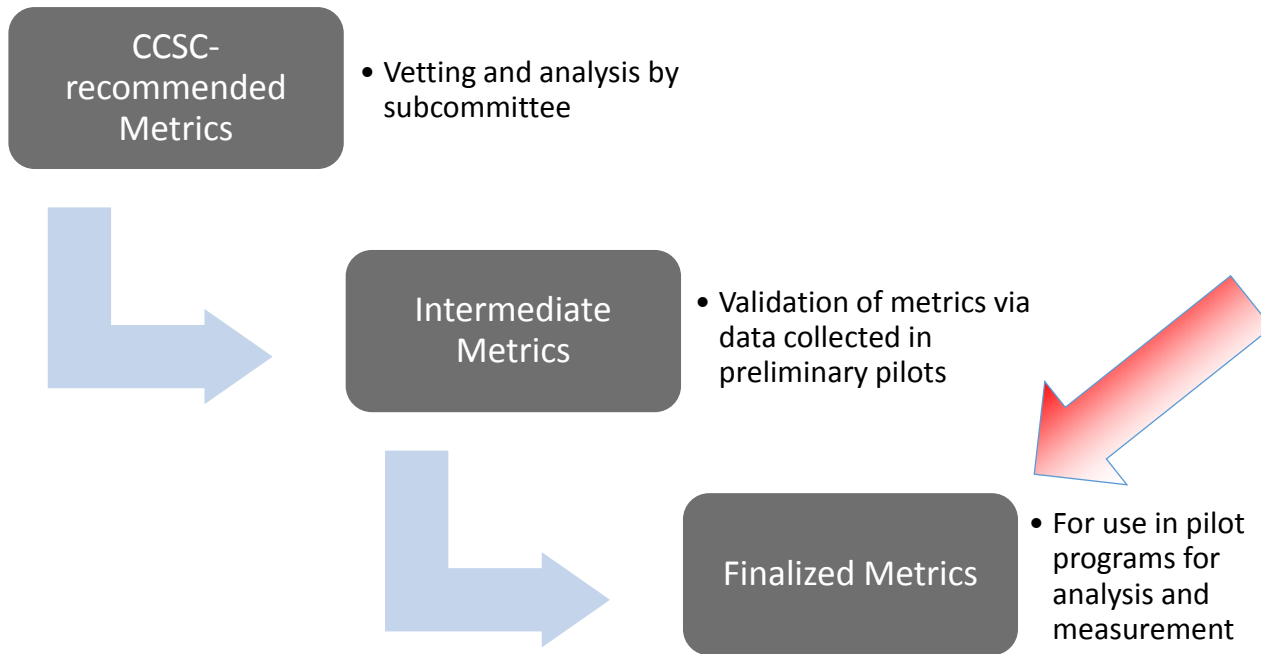
- Ongoing quality improvement (QI) and cost management initiative
- Driven by the insights of a prominent group of stakeholders:
 - Hemophilia treatment center (HTC) directors, clinicians, and administrators
 - Payer/managed care medical and pharmacy directors from a mix of large national and regional 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





Metric Development

CCSC METRIC DEVELOPMENT PROCESS



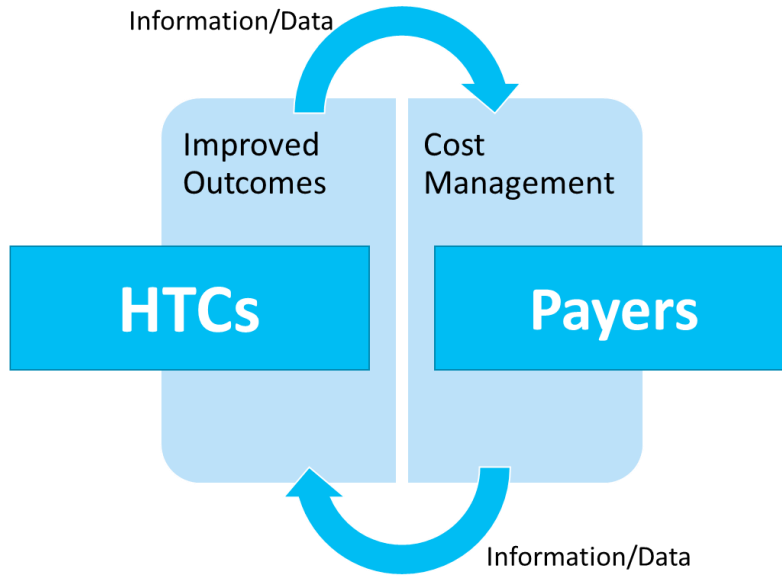
CCSC DEVELOPMENT OF FINALIZED METRICS

- 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, HTC'S, AND PAYERS CAN IMPROVE OUTCOMES AND MANAGE COSTS





METRICS FOR QUALITY IMPROVEMENT

CCSC-recommended Metrics for HTC 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 (\pm 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**
 - $\pm 10\%$ according to MASAC guidelines; payers desire $\pm 5\%$

Crucial for payers



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 1° or 2° 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



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