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AN ACTUARIAL STUDY OF HEMOPHILIA

IMPLICATIONS FOR COMMERCIAL AND MEDICAID MANAGED CARE PLANS

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EXECUTIVE SUMMARY: THE NEW RISK ENVIRONMENT

Much commentary on the dramatic changes to US healthcare focuses on technical insurance changes caused by healthcare reform such as reducing the number of uninsured, the creation of the Health Insurance Exchanges, and minimum loss ratios. Changes are also affecting the providers of care, notably Accountable Care Organizations, where new organizations are being created to meet the growing trend to reward hospitals, physicians and others for providing efficient and quality care—or to penalize them for inefficient and low quality care. These changes are occurring in the context of a weak economy, and the past growth of healthcare spending is broadly considered unsustainable.

State budget problems are also leading to changes in public programs. States continue to delegate more Medicaid beneficiaries, including the chronically ill, and more services to managed care organizations, and the potential for millions of newly eligible Medicaid recipients is leading to interest in forming new Medicaid HMOs.

This combination of insurance changes, new organizations, and financial pressures is creating new opportunities and new risks in the healthcare system. This paper is about the financial risks associated with hemophilia—a rare genetic disorder—in the context of these changes. Although hemophilia has unique characteristics, we believe the issues we address in this paper will find application as regulators, insurers, and provider organizations consider how they will financially manage the care for other rare and expensive medical conditions.

Hemophilia is a serious, potentially life-threatening condition. Apart from the potential complications involved in external bleeding episodes, individuals affected with hemophilia are at risk of poorly controlled internal bleeding which can cause pain and swelling. Over time, it can lead to permanent joint and muscle damage and chronic pain.[1] Bleeding disorders can lead to other serious complications, including anemia, brain hemorrhage, deep internal bleeding, infection, and neurological or psychiatric problems.[2,3]

Hemophilia A and B have a combined prevalence of about 13 cases per 100,000 male lives in a typical commercial population.[4] In our study of 2008-2011 healthcare claims, hemophilia A and B patients cost, on average, about $85,000 and $47,000 per year, respectively, compared to a cost of about $4,200 per year for the average person in a commercial population. Severe hemophilia A patients, by contrast, incurred average annual costs of about $160,000. Due to the very low prevalence rate of hemophilia, however, the estimated claim cost when spread over an average commercial population is only about $0.42 per member per month for both hemophilia A and B combined for medical and pharmacy costs—less than 0.2% of a typical commercial insurer's total budget. While most hemophilia patients exhibit relatively predictable costs over time, the overall population presents variability and thus unpredictability for forecasting costs. Some of the factors that contribute to this unpredictability are familial clustering, phenotypic variability (e.g., bleeding patterns, age, and response to therapy), the potential for surgery, and the development of inhibitors.

Since hemophilia is rare, there can be substantial fluctuation in the number of hemophilia patients different insurance organizations will have as members. The genetic basis of the disease leads to clustering in families, which can compound the variability around the number of hemophilia patients. This phenomenon, along with the great variability in cost per patient, creates challenges for risk-bearing organizations.

The risk of catastrophic costs is not new, and the insurance industry has managed that risk for decades. However, some of the techniques the industry used, such as lifetime limits and medical underwriting, will

no longer be allowed under healthcare reform. Reform regulation does include a set of newer tools (risk adjustment) and older tools (reinsurance) to manage these risks and to replace the techniques that will not be allowed. How well this set of tools will protect the financial stability of organizations assuming risk for hemophilia treatment or other high cost treatments is not clear.

This report identifies potential financial risks that, while affecting both small and large organizations, may be more difficult for smaller organizations to manage. These organizations may experience costs much different than the average due to the extreme variation in treatment costs per patient and the impact of familial clustering of patients. Of particular concern is risk delegated to smaller insurance entities, such as start-ups, or capitated provider systems. The latter tend to have relatively small numbers of members. We illustrate this by showing the financial impact that a single case of hemophilia A could have on a risk-taking entity. Take, for example, a provider organization with 5,000 commercial members at risk. Such a provider organization would typically budget some surplus to invest in physician incentives or system changes. The costs for one additional hemophilia patient could absorb a significant portion of its budgeted annual surplus for the year. Costs associated with a more severe hemophilia patient could absorb the entire annual budgeted surplus. Such deviations from budget could significantly disrupt the stability of the provider system. By contrast, an organization with a million commercial members would be able to spread the risk across a larger population. However, due to familial clustering, this type of organization could still experience significant variation in its financial results if it enrolls a family with multiple hemophilia patients.

Because of the difficulty in predicting the cost of hemophilia patients for an individual organization, some states have elected to carve out hemophilia patients or treatment costs from their managed Medicaid programs and pay for these services on a fee-for-service basis. Other pooling mechanisms, such as the risk adjustment and reinsurance programs that will be part of Exchanges, are other ways of managing risk. Although not perfect, these tools can help ameliorate this risk for smaller organizations.

Limitations

This report was commissioned by Baxter Healthcare Corporation, a global, diversified healthcare company that provides products for hemophilia treatment. The authors are Members of the American Academy of Actuaries and meet its qualification standards to issue this report. Milliman does not intend to endorse any product or to benefit any third party through this report; the report reflects the findings of the authors.

As with any forecast of healthcare reform, our work is based on many assumptions and cannot capture all influences. In particular, our analysis of hemophilia costs is based on recent historical experience, therapies, and cost levels. Future experience will vary from the projections presented in this report for reasons including random fluctuation. In addition, we present national average costs for typical populations and benefit designs, but the reader should note that considerable variation from the average results often occur in specific populations. We suggest that this report be distributed in its entirety, as material taken out of context can be misleading.

BACKGROUND

Hemophilia is one of the better-known rare medical conditions, perhaps due to its association with certain royal families, and is used as an example of hereditary diseases. Hemophilia is generally an inherited bleeding condition in which blood does not clot properly. People with hemophilia are missing a clotting factor in their blood, resulting in both the reduced ability stop normal bleeds and spontaneous bleeding.[5] Bleeding can be serious and potentially life-threatening. Individuals affected with hemophilia are also at risk of poorly controlled internal bleeding, resulting in pain and swelling. Over time, it can lead to permanent joint and muscle damage and chronic pain.[1] Bleeding disorder complications include serious, potentially life-threatening complications, including anemia, brain hemorrhage, deep internal bleeding, infection, and neurological or psychiatric problems.[2,3]

Hemophilia usually refers to hemophilia A (Factor VIII deficiency)[5] and hemophilia B (Factor IX deficiency)[6], but payers often include these along with Von Willebrand Disease (VWD) under the general category of bleeding disorders. Von Willebrand Disease is a bleeding disorder caused by a deficiency of a protein called Von Willebrand factor.[7] VWD is more common and usually less severe than hemophilia A and B.[8] In this report we use the term "hemophilia" to mean hemophilia A and hemophilia B, but not VWD as it is a different disease.

Hemophilia A and hemophilia B occur overwhelmingly in males. The gene that helps create clotting factor proteins is located on the X chromosome. Males who inherit an affected X chromosome from their mothers will have hemophilia. Females who inherit one affected X chromosome become "carriers", and can pass the affected gene to their children.[5] It is extremely rare for females to have hemophilia. In the data analysis described in this report, we excluded females coded with hemophilia, assuming that such codes were erroneous.

Hemophilia A and B are rare conditions. According to the Centers for Disease Control and Prevention, about 18,500 Americans are diagnosed with hemophilia, with approximately 14,500 hemophilia A patients and 4,000 hemophilia B patients.[9] About 1 in every 5,000 male babies are born each year with hemophilia A and 1 in 25,000 males babies are born with hemophilia B.[5,6] Of all hemophilia A and B cases, about 80% are hemophilia A.[9] Hemophilia is further classified by severity – mild, moderate, or severe – determined by the amount of clotting factor in a patient's blood.[5]

Diagnosis of hemophilia, its type and severity occurs through blood analysis. Desmopressin vasopressin analog (DDAVP) is the most common treatment for mild hemophilia A.[10] Desmopressin, usually taken by injection or nasal spray, helps the body release more factor VIII into the bloodstream.[11] For moderate and severe hemophilia, the main treatment is called factor replacement therapy. Factor replacement therapy entails infusing concentrates of clotting factor VIII (for hemophilia A) or clotting factor IX (hemophilia B) into a vein. Clotting factor concentrates can be derived from human blood (plasma derived) or can be in the form of recombinant products.[12] It is reported that half of all hemophilia patients were infected with human immunodeficiency virus (HIV) and/or hepatitis C virus (HCV) as a result of tainted blood products in the 1970s and early 1980s, resulting in increased mortality and morbidity of these patients.[13,14] Today, concentrates from human blood are treated to avoid the spread of these diseases.[13] Patients may be treated either on a regular basis with scheduled prophylaxis infusions or episodically at the time of a bleed (often referred to as "demand therapy").[15]

The development of an inhibitor, an antibody that destroys the clotting factor is the most serious complication involved in treating hemophilia.[16] The estimated lifetime incidence of these antibodies,

referred to as inhibitors, in patients with hemophilia A is between 20% and 30%.[17] That is, an individual patient has a 20% to 30% chance of developing an inhibitor over his lifetime, typically while an infant. The prevalence rate of inhibitors among hemophilia A patients is estimated at 2% to 9% while the prevalence rate of inhibitors among hemophilia B patients is 1% to 4%.[9,]

When inhibitors develop, doctors may use larger doses of clotting factor to stop a bleed. This is often ineffective, and frequently these patients have to rely on alternative treatment including bypass therapy.[16] Eradication of the inhibitor is an option which is accomplished by regular (daily or several times weekly) infusions of variable doses of factor VIII or IX to tolerize the immune system to these factors. This process is called Immune Tolerance Induction (ITI) treatment. This method is effective in 70% to 85% of patients with factor VIII inhibitors and may take one to two years to completely eradicate the inhibitor.[16] While ITI treatment is time consuming and costly, patients that are effectively treated can return to a normal regimen of factor replacement therapy.

The treatment for patients with or without inhibitors is guided by patient-specific factors such as bleeding patterns, age, activity level, and response to therapy.[18] In the US, there are 144 Hemophilia Treatment Centers (HTCs) dedicated to providing treatment and medical management of hemophilia.[19] Historically, about 70 percent of hemophilia patients receive treatment at these centers. A study conducted in 1984 found that the newly formed Hemophilia Centers were effective in reducing inpatient admissions, reducing absences from school or work, and reducing unemployment.[20] Soucie et al (2000) found that HTCs have had a significant effect in reducing the risk of mortality, about a 30% reduction, in hemophilia patients in the US.[14]

Hemophilia patients often incur substantial healthcare costs in comparison to the average commercially insured or Medicaid covered individual. Direct medical costs include factor replacement and other hemophilia treatment drugs, inpatient and ER procedures, outpatient care, laboratory tests, and use of medical devices. Indirect costs include the lost productivity of the patients and their respective families. In a recent claims-based study, Guh et al (2012) analyzed data on 1,164 men with hemophilia A and found that healthcare costs varied widely across patients, depending on their respective complications and treatment responses. In particular, the study reported that mean costs for inhibitor patients were 4.8 times higher than for patients without an inhibitor. In addition, adult patients with HCV or HIV infection incurred an average cost of treatment that was 1.5 times higher than that for patients without these infections.[21]

Some people with hemophilia, especially those with more severe symptoms, do not work or are not able to work and thus do not have access to health insurance coverage through employer plans. These people often rely on state Medicaid programs under “medically needy” eligibility categories. Medically needy programs are available in 33 states plus the District of Columbia for people who, after “spending down” their income for personal medical expenses have net income that meets Medicaid eligibility requirements.[22] Some states, recognizing the high variability in treatment costs and the rarity of the disease, “carve out” hemophilia drugs from the benefits covered by Medicaid managed care programs, which means they pay for hemophilia drugs directly instead of having the managed care organization bear that cost. Under carve out programs, hemophilia drug costs are covered by the state either on a fee for service basis or by a separate standalone program. Florida is an example of a state with a stand-alone hemophilia disease management program.[23] New Jersey is an example of a state that provides a separate payment to Medicaid managed care plans outside of the capitated payment.[24]

DESCRIPTIVE STATISTICS

The authors developed statistics on hemophilia prevalence and cost for commercially insured and Medicaid populations from recent administrative claims databases. By commercial insurance, we generally mean privately funded individual, small group, and large group coverage. We use the term "Medicaid" to indicate publically funded coverage under a Medicaid HMO or a state fee for service Medicaid program.

COMMERCIAL HEALTH PLAN DATA

We identified hemophilia patients in the *MarketScan*® commercial population database for 2008-2011 using the ICD-9 diagnosis codes for hemophilia and also using the presence of hemophilia-specific drugs. As describe in the Background section, we did not include females in the hemophilia cases. The Methodology section contains the identification logic.

Table 1 shows the hemophilia prevalence rates per 100,000 male members by hemophilia type for the four years of *MarketScan*® data examined. The total person-years for this period were approximately 56 million, with 27 million males.

TABLE 1
COMMERCIAL POPULATION
HEMOPHILIA PREVALENCE RATES PER 100,000 MALE INSUREDS BY AGE and TYPE

Age	Prevalence Rate per 100,000 Males	
	Hemophilia A	Hemophilia B
0-4	13.4	1.6
5-9	14.0	2.6
10-14	15.7	2.8
15-19	17.1	2.1
20-24	15.0	2.4
25-29	12.1	1.8
30-34	8.8	1.5
35-39	7.9	1.4
40-44	8.0	0.8
45-49	7.4	1.1
50-54	7.9	1.7
55-59	7.9	1.5
60-64	9.3	1.5
Total per 100,000 Males	11.0	1.8
Total per 100,000 Members	5.4	0.9

Source: Authors' analysis of *MarketScan® Commercial Database* 2008-2011

Hemophilia is typically diagnosed very early in life and is a chronic condition. The total observed prevalence rate of hemophilia A in commercially-insured males is 11.0 per 100,000. At the youngest ages, the prevalence is higher, between 13 and 17 per 100,000. By contrast, the CDC reports an incidence of 1 in 5,000 male births, or about 20 per 100,000, which is closer to our figures for younger ages. The lower overall prevalence in the commercial population compared to the CDC figure could be the result of

coverage lapses among commercially-insured hemophilia patients or mortality at older ages associated with HIV and HCV infections acquired from blood-derived factor treatments in the 1980s.

We observe a prevalence of 1.8 per 100,000 males for hemophilia B, which is about 14% of the total hemophilia A and B cases. Published sources also suggest hemophilia B accounts for about 14% of all hemophilia A and B cases, which is consistent with our results.

Figures 1 and 2 below show the prevalence rates for hemophilia A and B in male commercial members.

Figure 1
Prevalence of Hemophilia A Patients in Males
(Average in 2008 to 2011)

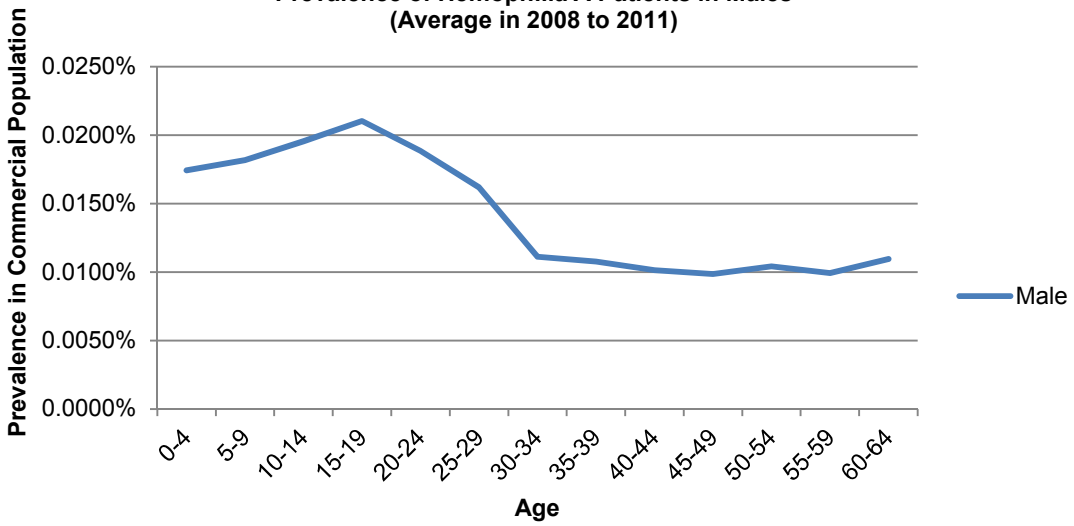
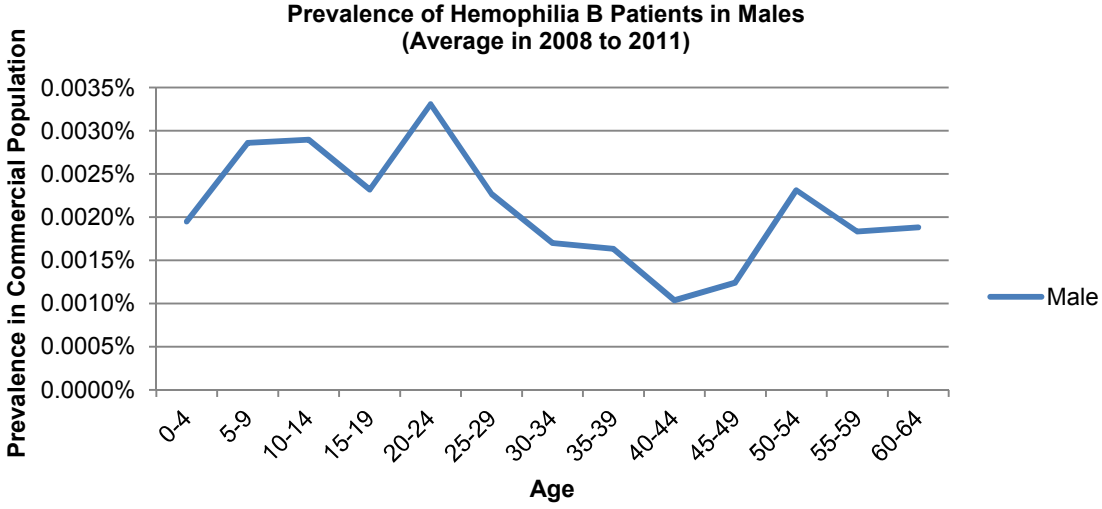


Figure 2
Prevalence of Hemophilia B Patients in Males
(Average in 2008 to 2011)



According to Figures 1 and 2, hemophilia prevalence in a commercial population declines with age. In addition to the patients shifting to Medicaid coverage, a contributor to this decline is mortality of the affected population, exacerbated by mortality from HIV/AIDS or other blood-borne pathogens, which were often transmitted through contaminated plasma-derived factor prior to blood screening.

Table 2 shows the annual cost of hemophilia treatment drugs by hemophilia type with respect to other claim costs. Factor administered during an inpatient stay is not included in the hemophilia drug costs below.

TABLE 2
COMMERCIAL POPULATION
AVERAGE ANNUAL ALLOWED CLAIM COSTS BY HEMOPHILIA TYPE

Medical Claims	Average Commercial Member	Hemophilia A	Hemophilia B
Hemophilia Drugs*	---	$64,153	$33,237
(percent of total)	---	76%	70%
All Other Services	$4,199	$20,585	$13,937
Total	$4,199	$84,738	$47,174

*Includes hemophilic factor, anti-inhibitor drugs, and other treatment drugs
Source: Authors' analysis of *MarketScan® Commercial Database* 2008-2011

Table 2 shows that, across the population of hemophilia patients, coagulation factor and other hemophilia treatment drugs make up about three-quarters of allowed claim costs for hemophilia A and B patients in 2008 to 2011. The average claim cost for all commercial members over the same period is $4,199 per year, implying that hemophilia A and B costs are about 20 and 11 times higher, respectively, than the average commercial member. It is also interesting to note that the allowed claim costs for hemophilia A and B patients, not including drugs, are still about 4.9 and 3.3 times higher than an average commercial member's claim costs.

It is important to keep in mind that the average claim costs presented in Table 2 and later in this report include hemophilia patients of varying severity. Because we used four years of data to identify hemophilia patients, some individuals, likely with very mild hemophilia, were identified as hemophilia patients but had no hemophilia drug claims in a particular year. Some of these patients had a hemophilia diagnosis code in one of the years of data but not in all four years. We estimate the annual claim costs for severe hemophilia A patients, about 50% of all people with hemophilia A, to be about $160,000. This is about 38 times the average member claim costs.

Table 3 further details the annual allowed claim costs by type of service.

TABLE 3
COMMERCIAL POPULATION
AVERAGE ANNUAL ALLOWED CLAIM COSTS BY HEMOPHILIA TYPE AND SERVICE CATEGORY

Service Category	Average Commercial Member Claims	Hemophilia A		Hemophilia B	
		Hemophilia Drug Claims*	Other Claims	Hemophilia Drug Claims*	Other Claims
Inpatient **					
Facility		$0	$9,104	$0	$5,143
Professional		$0	$556	$0	$241
Total	$1,065	$0	$9,661	$0	$5,384
Outpatient					
Facility		$3,898	$3,989	$1,574	$3,531
Professional		$33,441	$3,444	$20,647	$3,531
Total	$2,394	$37,339	$7,433	$22,220	$7,062
Pharmacy	$740	$26,814	$3,492	$11,017	$1,491
Grand Total	**$4,199**	**$64,153**	**$20,585**	**$33,237**	**$13,937**

*Includes hemophilic factor, anti-inhibitor drugs, and other treatment drugs
'** Cost of inpatient drugs are included in facility cost
Source: Authors' analysis of *MarketScan® Commercial Database* 2008-2011

We note the following:

- Average inpatient claims for hemophilia A patients ($9,661) are over nine times more than average commercial member inpatient claims ($1,065). The average inpatient claims for hemophilia A patients is almost twice that for hemophilia B ($5,384).

- Non-drug outpatient claims (both facility and professional) for the two hemophilia types are very similar, roughly $7,000 per year, which is about three times the average commercial member outpatient costs.

Table 4 shows the top 20 most frequent inpatient Diagnosis Related Groups (DRGs) and the admissions per 1000 patient years for hemophilia A and B patients in 2008-2011. For comparison purposes we also show the average frequency rates for all commercial members for these same DRGs. We note that the number one admission type is DRG 813 – Coagulation Disorders, which indicates that hemophilia is the primary reason for the inpatient admission. As expected, joint replacement and other bone procedures appear towards the top of the list. Almost all inpatient admissions for hemophilia A patients are coded with MCCs (Major Complications or Comorbidities), which will tend to increases the cost of hospital stays for these patients.

TABLE 4
COMMERCIAL POPULATION
TOP 20 DRGs FOR HEMOPHILIA A and B PATIENTS BY FREQUENCY

DRG	Description	Frequency (Admits/1000 Patient Years)	
		Hemophilia A & B Patients	Total Population
813	Coagulation disorders	17.0	0.1
314	Other circulatory system diagnoses w MCC	9.4	0.1
885	Psychoses	3.8	1.8
919	Complications of treatment w MCC	3.8	0.0
377	G.I. hemorrhage w MCC	2.8	0.0
793	Full term neonate w major problems	2.4	0.5
469	Major joint replacement or reattachment of lower extremity w MCC	2.3	0.0
133	Other ear, nose, mouth & throat O.R. procedures w CC/MCC	2.2	0.0
492	Lower extrem & humer proc except hip,foot,femur w MCC	2.1	0.0
391	Esophagitis, gastroent & misc digest disorders w MCC	2.1	0.1
604	Trauma to the skin, subcut tiss & breast w MCC	2.0	0.0
553	Bone diseases & arthropathies w MCC	2.0	0.0
871	Septicemia or severe sepsis w/o MV 96+ hours w MCC	2.0	0.3
392	Esophagitis, gastroent & misc digest disorders w/o MCC	2.0	1.5
897	Alcohol/drug abuse or dependence w/o rehabilitation therapy w/o	1.9	1.4
794	Neonate w other significant problems	1.8	1.9
602	Cellulitis w MCC	1.6	0.0
64	Intracranial hemorrhage or cerebral infarction w MCC	1.6	0.1
847	Chemotherapy w/o acute leukemia as secondary diagnosis w CC	1.5	0.3
795	Normal newborn	1.5	8.6

Source: Authors' analysis of *MarketScan*® commercial database, 2008-2011, Milliman Health Cost Guidelines (July 1, 2012), Inpatient Hospital Detail By MS-DRG (CMS v.29), Commercial Population, Loosely Managed Care

Hemophilia A and B patients have high average annual claim costs; however, the data suggests significant variation. To illustrate this, Table 5 shows the mean and 90th percentile annual claim costs by hemophilia type. (The 90th percentile is the amount above which 10% of patients had greater annual claim costs.)

TABLE 5
COMMERCIAL POPULATION
ANNUAL CLAIM COST MEAN AND 90th PERCENTILE

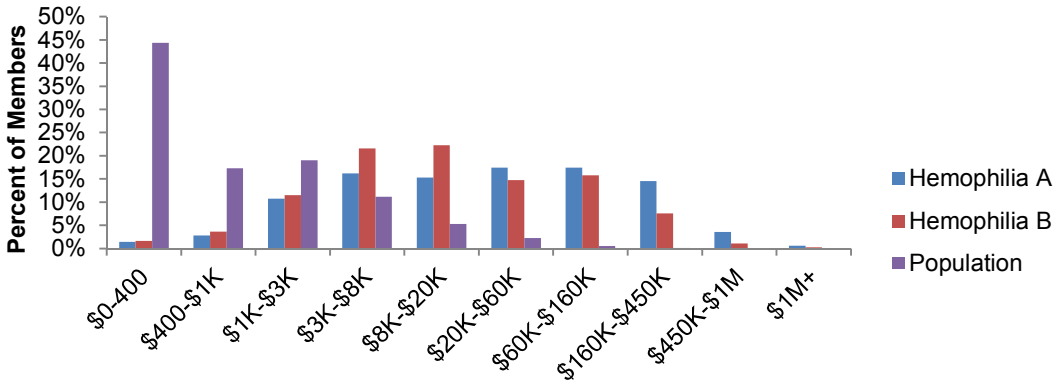
Type	Mean	90th Percentile
Hemophilia A	$84,738	$199,748
Hemophilia B	$47,174	$115,560
Total Commercial Population	$4,199	$8,404

Source: Authors' analysis of *MarketScan® Commercial Database* 2008-2011

According to Table 5, there is significant variation in claim costs for hemophilia A and B patients. The 90th percentile claim cost for hemophilia A patients is over $110,000 more than the average hemophilia A patient claim costs. For hemophilia B, the 90th percentile claim cost is over $60,000 greater than the average. In contrast, the 90th percentile claim cost for all commercial members is only about $4,000 more than the average.

As another metric in variation, the following graph depicts the percent of members experiencing annual claim costs in various dollar ranges. Note the spike in the $0-$400 range for the commercial population, as many members have no or very few claims in a given year. Furthermore, note the distribution of claims for hemophilia A and B patients. These distributions are quite dispersed and tend to be centered on higher ranges as compared to the commercial population. The implication here is that although hemophilia patients have high annual claim costs on average, it is difficult to tell where the claims will fall for a subset of the population in a given year.

Figure 3
Histogram of Annual Allowed Cost
Hemophilia Patients Vs. Commercial Population



MEDICAID DATA

Our Medicaid data source is a compilation of managed Medicaid data and fee-for-service data from four states, one state in the South, two in the Midwest, and one in the West. The data spans the years July 2006 to June 2009, with over 2.5 million person years over the three year period. We used this data source to develop Medicaid prevalence rates and average annual claim costs, although we note that the data may not be representative of other states or the nation as a whole.

We identified hemophilia patients and drug costs for the Medicaid population using the same methodology as our commercial data analysis. Table 6 shows hemophilia A and B prevalence rates per 100,000 Medicaid members.

TABLE 6
MEDICAID POPULATION
HEMOPHILIA PREVALENCE RATES PER 100,000 BY TYPE

	Prevalence Rate per 100,000	
	Hemophilia A	Hemophilia B
Total per 100,000 Males	21.2	3.0
Total per 100,000 Members	8.3	1.2

Source: Authors' analysis of Medicaid payer data (2006-2009)

We observed a combined prevalence rate for hemophilia A and B of 9.5 per 100,000 Medicaid members. The combined prevalence for hemophilia A and B among males is approximately 1 in 4100, somewhat higher than the overall US prevalence of 1 in 5000. These prevalence rates are 40%-50% greater than rates observed in the commercial population; however the relativities between hemophilia A and B remain similar, with about 12% of the cases identified as hemophilia B.

As with the commercial population, hemophilia A and B patients are on average higher cost than the typical member, with a large part of spending coming from hemophilia drugs. Table 7 shows the average allowed claim costs of these drugs with respect to total spending, by hemophilia type.

TABLE 7
MEDICAID POPULATION
AVERAGE ANNUAL ALLOWED CLAIM COSTS BY HEMOPHILIA TYPE

Medical Claims	Hemophilia A	Hemophilia B
Hemophilia Drugs*	$121,335	$36,043
(percent of total)	*(86%)*	*(48%)*
All Other Services	$19,506	$38,650
Total	$140,841	$74,694

*Includes hemophilic factor, anti-inhibitor drugs, and other treatment drugs
Source: Authors' analysis of Medicaid payer data (2006-2009)

As shown in Table 7, 86% of hemophilia A, and 48% of hemophilia B patient spending comes from hemophilia drugs. We also note that the average annual cost of hemophilia A patients observed in this Medicaid data source is approximately 66% greater than average observed in the commercial data (60% greater for hemophilia B). This may suggest that patients in Medicaid use more hemophilia factor.

Table 8 shows the annual allowed costs by hemophilia type by service category for the Medicaid population. As with the commercial population statistics, hospital facility claim costs and professional claim costs for hemophilia patients are significantly greater than for the average Medicaid member.

TABLE 8
MEDICAID POPULATION
AVERAGE ANNUAL ALLOWED CLAIM COSTS BY SERVICE CATEGORY

Service Category	Average Medicaid Member	Hemophilia A	Hemophilia B
Hospital Facility	$1,488	$13,900	$24,009
Professional	$1,217	$3,905	$11,033
Pharmacy (non-hemophilia)	$404	$1,700	$3,608
Hemophilia Drug Treatment	$14	$121,335	$36,043
Total	$3,124	$140,841	$74,694

Source: Authors' analysis of Medicaid payer data (2006-2009)

Table 9 shows the mean and 90th percentile annual claim costs for hemophilia patients by type for the Medicaid population.

TABLE 9
MEDICAID POPULATION
ANNUAL CLAIM COST MEAN AND 90th PERCENTILE

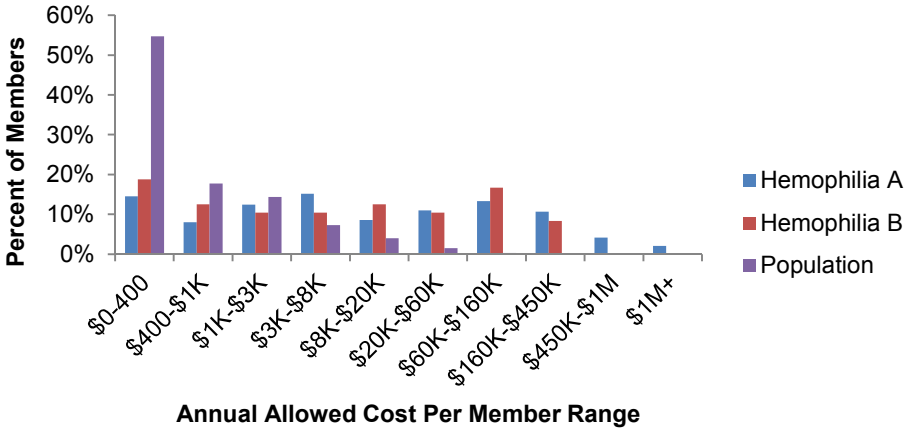
Type	Mean	90th Percentile
Hemophilia A	$140,841	$244,819
Hemophilia B	$74,694	$125,574
Total Population	$3,124	$4,915

Source: Authors' analysis of Medicaid payer data (2006-2009)

We note that the mean and 90th percentile annual costs for hemophilia A and B patients in the Medicaid data are significantly greater than in the commercial data. This suggests that patients covered under the Medicaid programs in these states use more hemophilia drugs. While the differences between the mean and the 90th percentile costs for hemophilia patients in the Medicaid population are somewhat less than the differences observed in the commercial data, the variance is still significant.

Figure 4, below, shows the distribution of costs by spend band for hemophilia A and B patients with respect to the general Medicaid population.

Figure 4
Histogram of Annual Allowed Cost
Hemophilia Patients Vs. Medicaid Population



Note that the general Medicaid population shows a significant portion of members falling in the $0-$400 range, with the percentage of members in higher spend bands trailing off rather quickly. Figure 4 shows hemophilia A and hemophilia B members distributed over a wide range of spend bands. This wide distribution for hemophilia patients leads to difficulty in predicting claim costs for these groups.

FINANCIAL IMPACT ANALYSIS: HIGH VARIABILITY IN NUMBER OF PATIENTS AND COST

Hemophilia has a complex impact on risk-bearing entities. Since hemophilia is rare, predicting the number of hemophilia patients for individual organizations becomes difficult, and the expected number of patients for organizations with small populations will be a fraction of a person. This may be intensified by family clustering, whereby the genetic nature of the disorder increases the conditional probability of a population having multiple members with hemophilia, and which may affect both small and large plans. Additionally, the phenotype of the particular patient with the disease significantly impacts the different levels of treatment necessary (bleeding patterns, age, response to therapy, need for surgery throughout lifetime and risk of developing inhibitors). Our analysis of costs associated with hemophilia patients suggests that they are more expensive on average, but a large variation in spending exists for a given patient. These factors taken in combination can amount to financial risk. This section illustrates these issues for a hypothetical Medicaid managed care plan.

The observed prevalence rates suggest that even small populations may have one or two hemophilia patients. For all populations except only the very large, the expected number of hemophilia patients can be hard to predict. Table 10 shows the mean and 90% confidence interval of hemophilia A or B patients as a function of Medicaid population size.

TABLE 10
MEDICAID POPULATION
MEAN AND 90% CONFIDENCE INTERVAL OF HEMOPHILIA PREVALENCE

Medicaid Membership	Number of Hemophilia A and B Patients					
	Mean		Lower 90% Confidence Limit		Upper 90% Confidence Limit	
	Hemophilia A	Hemophilia B	Hemophilia A	Hemophilia B	Hemophilia A	Hemophilia B
5,000	0.4	0.1	0.0	0.0	1.5	0.5
10,000	0.8	0.1	0.0	0.0	2.3	0.7
25,000	2.1	0.3	0.0	0.0	4.5	1.2
50,000	4.2	0.6	0.8	0.0	7.5	1.9
100,000	8.3	1.2	3.6	0.0	13.1	3.0

Source: Authors' analysis of Medicaid payer data (2006-2009)

The statistical mean is the expected number of patients having hemophilia A or B for a given size Medicaid population. For example, a group of 25,000 Medicaid members will likely have about 2 members with hemophilia A and 0 or 1 member with hemophilia B (0.3 on average). The upper and lower 90% confidence intervals describe the range of expected results with 90% confidence. For example a population consisting of 100,000 Medicaid members will likely have about 8 members with hemophilia A, but 10% of similar sized plans will have fewer than 4 or more than 13 hemophilia A patients.

In Table 10, the confidence intervals become smaller relative to the average as the population gets larger. The upper 90th percentile limit for the 5,000 member plan for hemophilia A (1.5) is about four times the expected number of patients with hemophilia A (0.4), whereas the upper limit for the 100,000 member plan (13.1) is less than double the expected number (8.3). The results are similar for hemophilia B patients. This illustrates that predictability of hemophilia improves for larger populations.

Variation in costs is driven by a host of factors. Patients require vastly different levels of treatment depending on the severity of the disorder. Additionally, the amount of factor required for successful treatment varies with body size. Hemophilia patients may develop inhibitors to factor treatment, a condition which is frequently temporary. Treatment of patients with inhibitors may require large amounts of factor

and other healthcare services due to the severe complications involved with this condition. As with other severe patients, compliance variability and different approaches to patient treatment also likely play roles.

An average hemophilia patient experiences significantly greater claim costs than the average member, according to our analysis. Tables 2 and 3 suggest the average patient with hemophilia A incurs costs that are 20 times the average commercial population member. The average patient with hemophilia B incurs costs that are 11 times the average commercial member. However, it is important to note that our methodology, as is true with any claims-based analysis, may overstate the true cost of hemophilia patients. As detailed in the methodology section, we first identify hemophilia patients and then consider their associated costs. Identification of hemophilia patients may be easier in years that they have had high cost or acute episodes; this may lead to the elimination of some low-cost hemophilia patients from the analysis. Such bias raises the observed average cost and reduces the observed prevalence.

Only very large populations will likely experience hemophilia costs similar to the commercial or Medicaid market averages due to the high variation in annual cost. Figures 3 and 4 show the wide range of annualized spending that hemophilia patients exhibit in the commercial and Medicaid populations, respectively. These graphs show a relatively level percent of members in each annual spend band from $1,000 to $450,000.

Financial implications for risk-bearing entities are driven by the high variation in prevalence and costs. Smaller risk-bearing entities may more readily experience a disproportionate share of hemophilia patients and/or a disproportionate share of hemophilia costs than larger entities. Family clustering may also exacerbate variability. Table 11 illustrates the impact of one extra hemophilia A patient on a Medicaid health plan's financial results, assuming the plan does not have access to any of the risk mitigation mechanisms described below, such as stoploss reinsurance, carve-outs, or risk adjustment.

TABLE 11
MEDICAID POPULATION
FINANCIAL IMPACT OF HEMOPHILIA CLAIM BY MEMBERSHIP

Medicaid Membership	Annual Premium* ($ million)	Profit Margin** ($ million)	% of Annual Expected Profit	
			Average Hemophilia A Claim	90th Percentile Hemophilia A Claim
5,000	$22.5	$0.2	62.6%	108.8%
10,000	$45.0	$0.5	31.3%	54.4%
25,000	$112.5	$1.1	12.5%	21.8%
50,000	$225.0	$2.3	6.3%	10.9%
100,000	$450.0	$4.5	3.1%	5.4%
1,000,000	$4,500.0	$45.0	0.3%	0.5%
2,000,000	$9,000.0	$90.0	0.2%	0.3%

*Assumes a premium of $375 per member per month
** Assumes profit margin of 1% of premium (contribution to surplus for not-for-profit plans)
Source: Authors' analysis of Medicaid payer data (2006-2009)

The above table illustrates the negative correlation between membership size and financial impact of an additional hemophilia patient. In this illustration, an average hemophilia A patient's claims will constitute 63% of the annual profit for a 5,000 member Medicaid plan. The claims for a higher-cost hemophilia A patient may constitute 109% of the expected profit margin, resulting in a financial loss. In other words, for a small plan, one additional, average cost, hemophilia patient could significantly impact the plan's profits (or contribution to surplus) and could result in a loss. Conversely, the financial impact of one additional hemophilia patient for a plan with a million members or more would be relatively small.

As previously discussed, variance is an issue for predicting both the number of hemophilia patients and their costs for an individual organization. Because of family clustering, a given plan may be more likely to be at either the low end or the high end of the expected number of hemophilia patients. Taken together, these two factors can lead to fluctuating financial results for even moderately sized plans.

FINANCIAL MANAGEMENT STRATEGIES IN THE NEW RISK ENVIRONMENT

Insurance companies and managed care organizations have used a number of tools to manage the risk of adverse claims experience, which are described below. We also examine changes in the commercial health insurance market as a result of national healthcare reform, most notably the development of insurance exchanges for the individual and small group markets, and recent trends in the managed Medicaid market.

TRADITIONAL RISK MANAGEMENT MECHANISMS

Delegation or Carve Out

An effective way for insurance organizations to manage the risk associated with a particular healthcare service or population is to delegate it or carve it out. For example, some health insurers choose to carve out mental health benefits or radiology services since there are nationwide behavioral health specialty vendors that may manage these services more effectively and are willing to take capitated payments. Another form of delegation occurs with Accountable Care Organizations (ACOs), where the ACO may assume risk for members associated with its providers. Carve out programs for hemophilia related services are not available in the commercial market, but as discussed above, some states carve out hemophilia treatment services from their managed Medicaid benefit programs.

Medical Underwriting

Medical underwriting by health insurers will not be allowed beginning 2014 under the terms of the Patient Protection and Affordable Care Act (PPACA or ACA), but we include discussion here because it helps explain historical dynamics.

Over the past century, health insurers have often used medical underwriting to manage risk, especially in the individual and small group market. Subject to varying state restrictions, the medical underwriting process requires the insurance applicant to answer questions about his or her current health conditions, health history, and lifestyle. Applicants for individual health policies could be required to submit a physical exam and lab tests. Based on this information, insurers could choose to offer a policy or reject the application. For those meeting the minimum standards of the insurer but with less than the desired health status, companies could issue the policy at substandard rates (that is, higher premium) or exclude coverage of certain medical conditions (called pre-existing conditions exclusions). Thus, in many states, before the ACA, persons with hemophilia who do not have access to health insurance coverage from a large employer plan might not be able to get health insurance coverage or might not be able to afford the high premium rates.

Benefit Design

Benefit design has been a tool that insurers have used to limit their exposure to higher cost medical conditions and discourage members with certain conditions from enrolling. The use of annual limits and lifetime limits places a cap on the insurer's financial loss. Often, these dollar limits coupled with higher cost sharing for certain services discourages anti-selection. How the ACA affects limits is described below.

Reinsurance

Reinsurance is another mechanism used by insurers to mitigate the risk of excessive claims, either the risk that aggregate claims for the entire pool of members exceed a defined budget or the risk of large individual outlier claims. For purposes of this discussion, we will focus on the latter form of reinsurance, called

individual stoploss reinsurance or excess loss reinsurance. Insurers may purchase reinsurance policies from another insurer or from a specialty reinsurance company.

Insurance organizations (including provider organizations who take risk) purchase stoploss reinsurance to protect themselves against the risk of one or more members with catastrophic claims. As seen above, one or two members with claims of $1,000,000 or more can have a significant impact on the financial results for small companies. While some expensive patients are always expected, small companies may experience some years with no catastrophic cases and other years with multiple catastrophic cases. Thus, the purpose of reinsurance is to smooth out these random fluctuations. Organizations with a larger pool of members experience smaller random fluctuation, as their experience tends toward the average. Therefore, most large companies do not purchase reinsurance for this purpose.

While there are many variations, a typical stoploss reinsurance policy will cover claims for an individual insured member during a 12-month period that exceed a specified dollar amount—called the attachment point. Attachment points for the smallest organizations may be $100,000, meaning that the organization is responsible for the first $100,000 of an individual's claims and then the reinsurer pays the excess, usually up to a set maximum such as $1,000,000. This form of risk management works well when the risk is random. Results will even out over time, and small companies are willing to pay more than the average catastrophic cost (the reinsurer's premium includes the average cost plus profit margin and administrative load) for more predictable financial results.

In the case of hemophilia, the risk is not random as it is for accidents, cancer chemotherapy or some other high-cost conditions. Some member with severe hemophilia A may incur very high costs every year. They may require large amounts of hemophilic factor and also develop other complications. Likewise, some hemophilia patients may incur catastrophic claims any time they have a bleeding incident, such as a car accident or surgical procedure. Private reinsurers usually underwrite for large ongoing claims and medical conditions, and thus will charge higher premium rates after it is known that a hemophilia member is in the group. However, the limited duration of the most expensive therapies, if understood by the reinsurer, may ameliorate rate hikes after very high cost cases are resolved.

As discussed below, the ACA has established a temporary reinsurance program for individual policies sold through the Health Insurance Exchanges beginning in 2014. The main purpose of this program is to help stabilize individual premium rates during the initial years of the individual mandate provision. The reinsurance program will not be available after 2016.

Holding Additional Capital

All health insurers and HMOs are required by state law to hold a minimum amount of capital and surplus (or net worth). In most states, there are no specific requirements for ACOs. The required amount of net worth varies by state but is usually defined by statute – either a "risk-based capital" methodology or some percentage of annual premiums or claims. The purpose of surplus is to create a high degree of certainty--perhaps with 99%-- that the organization can survive through unexpected losses, such as catastrophic losses, inadequate rates, or business interruption. A company knowing that it covers a hemophilia patient or other known high-risk patients should examine the possibility of high claims in one or multiple years, and as a result, may need to hold additional capital in the event that future large claims coincide with other negative events.

COMMERCIAL MARKET REFORMS

The Patient Protection and Affordable Care Act (PPACA or ACA), passed in 2010 requires significant changes to the commercial health insurance market, most of which will be implemented by the beginning of 2014. The primary goal of the ACA is to reduce the number of people who cannot obtain or cannot afford health insurance. Some of the reforms that have already been implemented and have had important implications to the current landscape are:[25]

- Prohibition of denying coverage to children based on pre-existing medical conditions
- Elimination of lifetime dollar limits on essential benefits
- Extending coverage to adult children up to age 26

For hemophilia patients, these changes mean that more children and young adults can continue to be covered under individual and small group plans and coverage cannot be denied because of their condition. The elimination of the lifetime dollar limit will allow more hemophilia patients to be covered by private health insurance rather than transferring to Medicaid.

Also of interest to hemophilia patients, the ACA requires private, non-grandfathered individual and small group health insurance plans to offer a minimum set of benefits, under the "essential health benefits rule" (EHB Rule) beginning in 2014.[26] The ACA established ten general categories of EHBs (for example, hospitalization, prescription drugs, preventive care), and each state has published a benchmark plan that services as the minimum standard for individual and small group health plans in its respective state. Hemophilia drugs and treatments are covered as medical benefits under most health insurance plans and would be considered part of the EHBs.

There is a growing trend for health insurers to move hemophilia and other specialty drugs to their plans' pharmacy benefits which, under the ACA EHB Rule, would make these drugs subject to the EHB formulary requirements. Hemophilia factor drugs could be included under the United States Pharmacopeia (USP) therapeutic category of Blood Products/Modifiers/Volume Expanders and USP class of Coagulants or Blood Modifiers. Beginning in 2014, individual and small group plans must offer at least the same number of drugs in each USP therapeutic category and class as the state's benchmark plan, or one drug if the benchmark plan does not cover the particular category and class.[27] While formulary restrictions generally have not presented issues for coverage of hemophilia drugs in the past, health plans may use the EHB formulary rules as a way to cover only chosen brands of hemophilia factor drugs.

Perhaps the most significant provisions of the ACA are scheduled to be implemented in 2014. Commercial health insurance companies and managed care organizations will not be able to use medical underwriting in the individual and small group market to deny coverage, exclude conditions or increase premium rates. Insurers will be required to accept all applicants regardless of current health status or history. In addition, all individuals are required to show evidence of health insurance coverage or face tax penalties and certain employers that do not provide health insurance benefits will also face penalties.

Individuals and small groups will be able to access the health insurance market through state or federally-run insurance exchanges where they will be able to shop for and compare benefit plans and premium rates. There are currently 18 states planning to run their own insurance exchanges and 7 other states are planning some form of partnership with the federal government.[28] The remaining states will default to the federally run exchange. The new pool of insured lives is expected to be a mix of healthy individuals who had not purchased insurance because they did not need it and individuals with expensive medical conditions who did not qualify for insurance previously. Some plans may attract a greater proportion of healthy, low cost members, while other plans may attract the higher cost members. Recognizing a need to

level the playing field given that insurers will no longer be able to underwrite for medical conditions, the government is establishing three risk management mechanisms known as the three R's—risk adjustment, risk corridors, and reinsurance.

The following table briefly describes the key elements of these programs.

KEY ELEMENTS OF COMMERCIAL EXCHANGE RISK MANAGEMENT PROGRAMS[29]

Program	Temporary or Permanent	Main Purpose	Applicability
Risk Adjustment	Permanent	To mitigate adverse selection by assessing charges on plans with lower risk members and transferring funds to plans with higher risk members. Plans will no longer have an incentive to avoid unhealthy individuals if the risk adjustment formula accurately reflects the risk of specific medical conditions.	All fully insured non-grandfathered individual and small group plans, both on- and off- of the Exchange.
Reinsurance	Temporary (2014 – 2016)	To mitigate risk for individual market plans covering persons with high medical costs who previously did not qualify for insurance and to make these plans more affordable.	Individual non-grandfathered plans will be eligible to receive payments. All health plans and self-funded employer plans will make contributions to the program, $5.25 PMPM in 2014.
Risk Corridors	Temporary (2014 – 2016)	To protect qualified health plans, both individual and small group, from misestimating projected expenses due to so many newly insured individuals joining the plans.	Individual and small group qualified health plans will be eligible to receive payments from the federal government if their actual expenses are over 3% greater than projected. They will remit a portion of their "savings" if actual expenses are over 3% less than expected.

These three risk management mechanisms will address some of the additional risk associated with higher cost conditions, including hemophilia, but not all. Risk adjustment provides relief to insurers for members whose expected healthcare costs are greater than the average. Under a typical program, each member of a plan is assigned a risk score based on his age, gender, and the ICD-9 diagnosis codes associated with his actual claim history. An average risk score is calculated for the plan's total membership, and the plan receives a premium rate equal to the average rate times the plan's risk score. For example, a plan having a risk score of 1.2 would receive a premium rate or capitation that is 20% higher than the total market for that product. Many states also use risk adjustment programs for their managed Medicaid plans.

The methodology used to compute the factors for assigning risk scores is based on average costs for members with specific health conditions. Therefore, risk adjustment can be expected to alleviate the cost of the average hemophilia patient but will not fully compensate for the most severe cases. The final rule issued by the Department of Health and Human Services (HHS) on March 11, 2013 shows the risk scores associated with hemophilia under the federal risk adjustment formula. All states except Massachusetts, which is planning to run its own risk adjustment model, will be using the HHS risk adjustment model.

Depending on the level of health plan benefits, adult members with hemophilia will receive a factor of 49.3 to 49.8 added to their risk score, while child members will receive a factor of 45.5 to 46.4. Another consideration is how hemophilia patients will be identified. The final rule indicates that the program will only use diagnosis codes on hospital and professional claims to assign risk scores, although subsequent guidance seems to allow diagnoses based on chart reviews and health risk assessments.[30] Hemophilia patients obtaining their drugs through a specialty pharmacy program may not be identified unless they have a hemophilia ICD-9 diagnosis code from one of these sources. Our commercial data analysis revealed members with significant pharmacy claims for hemophilia factor products and no other hospital or physician claims with hemophilia diagnoses.

The risk corridor program is a transitional program intended to provide protection for qualified health plans for underestimating the overall medical costs of the new members. Plans will be reimbursed for a portion of their claims that exceed 103% of their total expected claims costs. Plans with favorable experience will have to share their gains with the federal government if the gains exceed 3% of the expected claim costs. The risk corridor program will be in place only for 2014 through 2016, since the theory is after three years, the newly insured population's claim costs will be fully reflected in the commercial insurance pool.

The reinsurance program is also a transitional program for individual health plans. The purpose of this program is to stabilize individual market premium rates during the first three years of the law's implementation. All commercial health insurance plans and self-insured employer plans will be required to make contributions to a pool to cover the excess costs of high risk individuals covered. The reinsurance provisions for 2014 will reimburse individual insurers 80% of an individual's annual claim costs above an attachment point of $60,000, subject to a cap of $250,000. This program may address some of the risk of hemophilia patients but will gradually phase out and expire at the end of 2016.

RECENT TRENDS IN THE MEDICAID MARKET

In addition to the commercial market reforms discussed above, the ACA includes provisions that will increase the availability of health insurance coverage through expanding Medicaid eligibility. Beginning in 2014, states that choose to participate in the Medicaid expansion will increase eligibility thresholds from 100% to 138% of the Federal Poverty Level (FPL). Currently 24 states and the District of Columbia have announced plans to participate in the Medicaid expansion.[31] States that do not participate risk leaving limited options for those between 100% and 138% FPL, as these persons will also not have access to subsidies meant to lower the cost of purchasing insurance on the commercial exchanges. Although the federal government plans to finance most of the cost of the expansion through 2020, some states choosing not to participate site financial hardship.

A great deal of recent regulatory activity involves dual eligible beneficiaries, those individuals eligible for Medicare and Medicaid benefits. In the past, there has been little incentive for states to enroll dual eligibles in managed care plans because Medicare is the primary insurer. However, many states are working with CMS to participate in demonstration programs that will develop managed care programs for the dual eligible population on a fully-integrated basis so that the states and the federal government can share in any savings.

States working under increasingly tight budgets continue to transition more Medicaid recipients to lower cost managed care programs, a process that began decades ago. Some states have instituted mandatory enrollment in private managed care plans while others have voluntary programs where beneficiaries can choose either traditional or managed care programs. As of 2011, over 70% of Medicaid enrollees nationwide receive Medicaid benefits through private MCOs, with twenty-two states having more than 80% of Medicaid enrollees in managed care.[32] According to the Kaiser Family Foundation's 50-State Medicaid

Survey, as of 2010 about two thirds of all Medicaid managed care enrollees are in Medicaid only health plans. The average size of these plans varies greatly, but it is not uncommon for some local, Medicaid only plans to have 10,000 or fewer covered lives.[33]

Another recent trend in the Medicaid market is for states to delegate pharmacy benefits to managed care programs. Historically, the state Medicaid programs have received higher rebates and lower prices from drug manufactures than private health insurance plans. In order to take advantage of these rebates, states Medicaid programs have generally retained pharmacy benefits while delegating medical benefits to the managed Medicaid plans. The ACA required drug manufacturers to pay the same higher level rebates and to charge the same prices to Medicaid managed care plans, and as a result, many states are shifting their fee for service carve out pharmacy programs to the managed care plans.[34]

As demonstrated earlier, small MCOs may need to consider how to protect themselves from high cost variation such as associated with hemophilia A and B patients. With the trend for states shifting responsibility for pharmacy benefits to the managed care programs, risk management programs including private reinsurance may be helpful.

APPROACHES TO MANAGING RISKS

One approach to help organizations manage these risks is for states to carve out high cost drugs, for example, the hemophilia drug benefits, from the managed Medicaid benefit package. The state would reduce the premium amounts paid to the organization by an appropriate amount. Several states have adopted this approach. Pooling of hemophilia claims is another mechanism that could protect all MCOs from either a very large hemophilia claim or multiple hemophilia claims. Under this type of system, the claims of all hemophilia patients would be pooled and those MCOs experiencing higher than average claims would receive a refund from the pool and those experiencing lower than average claims, or no claims, would pay into the pool.

Many states have their own reinsurance programs for managed Medicaid plans. There is a great deal of variation from state to state with respect to how these programs work, for example, what services are covered under the reinsurance, whether the program is voluntary or mandatory, and how much protection is afforded. Managed Medicaid plans should assess the need for private reinsurance in addition to the state coverage, if available.

Another method used by states is to adjust for varying health risks between managed Medicaid plans through risk adjustment. As noted above, a risk adjustment system tries to account for the health risk of covered individuals by adjusting premium rates or capitation rates for the average cost of certain conditions. This system does not eliminate risk as the cost of hemophilia care for some individuals can be many times more than the average cost.

METHODOLOGY

Identification and classification of hemophilia patients is an integral part of this analysis. We use a combination of ICD-9 diagnosis codes and hemophilia drug codes (both J Codes and NDC codes) to identify hemophilia patients. We excluded female patients in identifying Hemophilia A and B due to the genetic unlikelihood of females having these diseases. We used the *Medi-Span* database to identify NDC codes associated with hemophilia treatment, and we relied on Baxter's clinical experts to cross-walk each NDC to the appropriate category (A, B, Inhibitor).

For the commercial data analysis, we looked at the four years (2008-2011) of *MarketScan*® data combined rather than each year separately to identify hemophilia patients. Combining three years also resolved the under-diagnosis issue that appears because some patients do not generate any claims with hemophilia diagnoses in one year. We used the same approach for the Medicaid data analysis, combining the three years of claims experience July 1, 2006 to June 30, 2009 to identify patients.

The following is a list of the ICD-9 codes associated with hemophilia.

ICD-9 Code	Description
286.0	Congenital factor VIII disorder (Hemophilia A)
286.1	Congenital factor IX disorder (Hemophilia B)

The following tables list the NDC generic names (for hemophilia drugs provided through a specialty pharmacy program) and J Codes (for hemophilia drugs provided in the physician office setting or hospital outpatient setting) that were used to identify hemophilia patients and their hemophilia drug costs.

NDC Generic Name (Various Units)
Antihemophilic Factor (Human) for Injection
Antihemophilic Factor (Recombinant) for Injection
Antihemophilic Factor rAHF-PFM for Injection
Antihemophilic Factor Recombinant PAF for Injection
Antihemophilic Factor/VWF (Human) for Injection
Antiinhibitor Coagulant Complex for Injection
Coagulation Factor IX (Recombinant) for Injection
Coagulation Factor IX for Injection
Coagulation Factor VIIa (Recomb) for Injection
Desmopressin Acetate Injection
Desmopressin Acetate Nasal Solution
Desmopressin Acetate Nasal Spray Solution
Factor IX Complex for Injection

J/Q Code Descriptions	
Code	**Description**
Q0187	Factor VIIa (coagulation factor, recombinant) per 1.2 mg
Q2022	Von Willebrand factor complex, human, per iu
J2597	Injection, Desmopressin Acetate, per 1 mcg
J7185	Injection, Factor VIII (antihemophilic factor, recombinant) (Xyntha)
J7186	Injection, antihemophilic factor VIII/Von Willebrand factor complex (human)
J7187	Injection, Von Willebrand factor complex (humate-p), per iu vwf:rco
J7188	Injection, Von Willebrand factor complex, human, iu
J7189	Factor VIIIa (antihemophilic factor, recombinant), per 1 microgram
J7190	Factor VIII (antihemophilic factor, human) per i.u.
J7191	Factor VIII (antihemophilic factor (Porcine)), per i.u.
J7192	Factor VIII (antihemophilic factor, recombinant) per i.u., not otherwise sp.
J7193	Factor IX (antihemophilic factor, purified, non-recombinant) per i.u.
J7194	Factor IX, complex, per i.u.
J7195	Factor IX (antihemophilic factor, recombinant) per i.u.
J7197	Antithrombin III (human), per i.u.
J7198	Anti-inhibitor, per i.u.
J7199	Hemophilia clotting factor, not otherwise classified

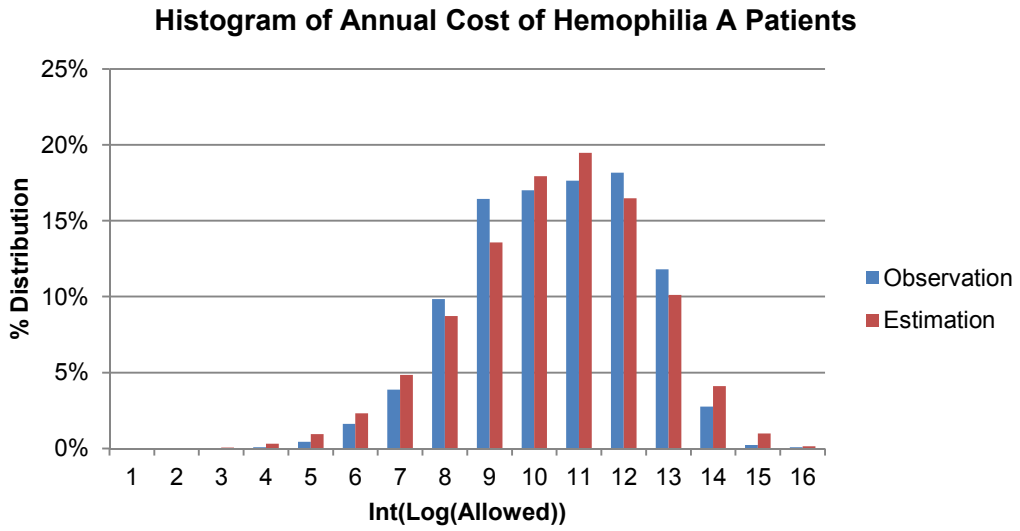
The identification hierarchy is as follows:

1. A male member is a hemophilia A patient if he has:
    a. one inpatient claim, one emergency room claim, or two evaluation and management (E&M) claims with diagnosis code 286.0, or
    b. a claim with an NDC or J code we have tied to hemophilia A, or
    c. one E&M claim with diagnosis code 286.0, and either an anti-inhibitor drug claim or a drug claim (such as DDAVP) which is indicated for treating both hemophilia A and VWD.
2. A male member is a hemophilia B patient if he is not a hemophilia A patient and has:
    a. one inpatient claim, one emergency room claim, or two E&M claims with diagnosis code 286.1, or
    b. a claim with an NDC or J code we have tied to hemophilia B, or
    c. one E&M claim with diagnosis code 286.1 and an anti-inhibitor drug claim.

For general commercial and Medicaid population claim probability distributions, we assumed log normal distributions. However, we could not assume log normal distributions to estimate the CPDs for hemophilia patients because their claims are skewed to the right (higher cost levels). In order to estimate the 90th percentile claims and claim probability distributions (CPDs) of hemophilia patients, specifically the information provided in Tables 5, 9, and 11 and Figures 3 and 4, we assumed a Weibull distribution scaled to the observed average cost of hemophilia patients. Generally, the probability distribution of a Weibull distribution of a random variable x can be written by two parameters of κ and λ as shown below.

$$f(x;\lambda,k) = \begin{cases} \frac{k}{\lambda}\left(\frac{x}{\lambda}\right)^{k-1} e^{-(x/\lambda)^k} & x \geq 0, \\ 0 & x < 0, \end{cases}$$

The chart below demonstrates how the Weibull distribution fits the observed values for the commercial hemophilia A patients.



DATA SOURCES

Truven Health Analytics MarketScan® Commercial Claims Database (MarketScan®) is an annual medical and prescription drug database that includes private sector health data from approximately 100 payers. The dataset contains more than 35 million commercially insured lives. It represents the medical experience of insured employees and their dependents for active employees, early retirees, COBRA continues and Medicare-eligible retirees with employer-provided Medicare Supplemental plans.

Medi-Span consists of the Master Drug Data Base v2.5 (MDDB®), the Medical Conditions Master Database ™ (MCMD), and the Drug Indications Database (DID). The MDDB is a comprehensive data source containing descriptive and pricing information for more than 150,000 drug products. The MCMD contains information about medical conditions, including name, type, and populations affected. MCMD also enables the user to cross reference ICD-9 and Disease codes to medical conditions. The Drug Indications Database offers deeper insight into medical conditions found in MCMD by identifying drugs available to treat them.

The Medicaid data source is a compilation of managed Medicaid data and fee-for-service data from four states, one state in the South, two in the Midwest, and one in the West. The data spans the years July 2006 to June 2009, with more than 2.5 million person years of coverage over the three year period.

We relied on information provided by Baxter Healthcare as to the hemophilia disease type associated with each NDC code. We used this information to refine our methodology for identifying hemophilia patients who did not have an inpatient, emergency room or E&M claim with a 286.0 or 286.1 diagnosis code but did have one or more claims for hemophilia specific drugs.

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