2012 Prevalence and Cost of Coagulation Factor Treatment for Hemophilia and von Willebrand's Disease Among 10 Million Commercially Insured Members

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Background

- Hemophilia A (Hemo A) and B (Hemo B) respectively denote congenital deficiencies of coagulation factor VIII (F8) or factor IX (F9) that vary in severity among individuals. As von Willebrand's Factor (vWF) acts as a carrier for F8, congenital deficiency of vWF (von Willebrand's Disease, vWD), can result in low F8 levels and similar bleeding propensity.
- Treatments include:
  - Continuous prophylaxis — regular infusions of the deficient factor (for example, F8 three times/week for Hemo A or F9 two times/week for Hemo B) — for individuals with hemophilia who have a severe coagulation factor deficiency;
  - "On-demand" factor infusions for individuals with hemophilia or vWD who are bleeding or preparing for surgery;
  - "Autologous replacement therapy" — desmopressin (DDAVP injection or nasal spray) can cause a transient rise in F8 and vWD factor for some individuals with moderate or mild hemophilia A or vWD who are bleeding or preparing for surgery; and
  - Non-pharmacotherapy usual approaches to bleeding or surgery for many individuals with only mild factor deficiency.
- Many individuals treated with factor products develop inhibitors (antibodies reactive with factor products) that diminish the factor effect. These individuals may be treated by increasing factor doses, use of the bypassing products Factor VIIa (NovoSeven RT®) or anti-inhibitor complex (FEIBA NF®), and/or efforts to induce immune tolerance through a treatment course using continuous high doses of factor.
- Most health insurers cover coagulation factor products through the medical benefit, but an increasing number are choosing to shift coverage to the pharmacy benefit.

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Objective & Purpose

- To determine, using administrative claims data: the prevalence of coagulation factor treatment for hemophilia or vWD, the distribution of annual factor cost for treated members, and variables associated with variation in cost among individual members.
- The underlying purpose is to help with development of strategies through which health plans seek to improve management of members with hemophilia or vWD.

Methods

- From 10 million commercial members younger than 65 years insured by 11 health plans, all were selected who were continuously enrolled in 2012, with complete medical and pharmacy claims data.
  - Members with one or more claim for F8, F8/vWF, or a bypassing product, were categorized as Hemo A if they were male and had two or more medical claims with the diagnosis code for Hemo A
  - Members with one or more claim for F8, F8/vWF, or a bypassing product, were categorized as vWD if they were either male or female and had two or more medical claims with the diagnosis code for vWD
  - Members with one or more claim for F9 or a bypassing product were categorized as Hemo B if they were male and had two or more medical claims with the diagnosis code for Hemo B
- Total factor cost was defined as plan plus member payments for all coagulation factor product claims.
- To subcategorize Hemo A and Hemo B members by whether or not observed factor cost was consistent with continuous prophylaxis, the expected cost of such therapy by age was estimated using NHANES weight by age data[1] and a published survey of hemophilia dosing practice.[2]
  - For Hemo A, the expected lower and upper cost by age was defined as the twenty-fifth and seventy-fifth percentiles of body weight by age from NHANES, respectively, times 20 units F8/kg (estimated low end of cost range) or 50 units/kg (estimated high end), times three/week times 52 weeks times the median payment per unit of F8 on all 2012 claims.
  - For Hemo B, this estimate used 50 units F9/kg as the estimated low end of cost range and 100 units/kg as estimated high end, delivered two/week (F9 has a longer half life than F8) and the median payment per unit of F9.

Figure 1. Hemophilia A: 2012 total factor cost by member plotted by member's age and whether or not treatment included a bypassing agent (Factor VIIa or anti-inhibitor complex). Dashed lines show estimated approximate upper and lower range of cost for continuous prophylaxis with Factor VIII

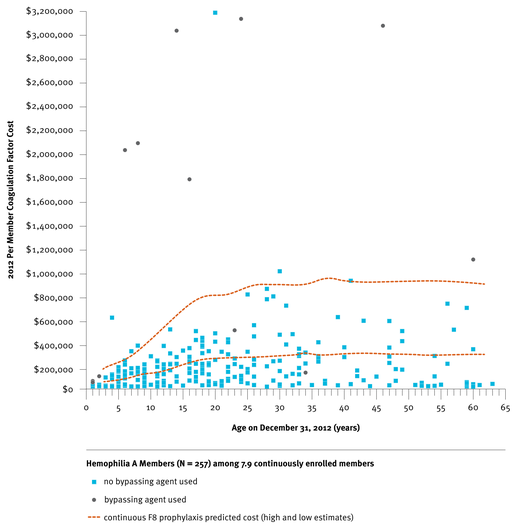

Figure 2. Hemophilia B: 2012 total factor cost by member plotted by member's age and whether or not treatment included a bypassing agent (Factor VIIa or anti-inhibitor complex). Dashed lines show estimated approximate upper and lower range of cost for continuous prophylaxis with Factor IX

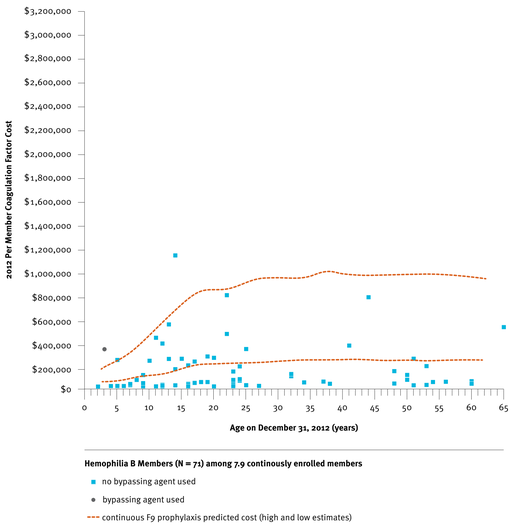

Table 1. Prevalence of any coagulation factor treatment (N members treated per 100,000 total members) by age groups

	Prevalence of Coagulation Factor Treatment (N per 100,000 members) by Age Groups (Years)													
Diagnosis	**00–04**	**05–09**	**10–14**	**15–19**	**20–24**	**25–29**	**30–34**	**35–39**	**40–44**	**45–49**	**50–54**	**55–59**	**60–64**	**Total**
Hemophilia A	7.56	7.23	5.29	6.46	5.04	3.53	2.88	1.48	1.29	1.46	0.88	1.82	0.60	**3.26**
Hemophilia B	1.14	1.71	2.05	1.82	1.91	0.59	0.51	0.33	0.29	0.53	0.63	0.14	0.60	**0.90**
von Willebrand's Disease	0.92	1.52	1.88	0.50	1.22	–	0.51	0.33	0.86	0.13	–	0.84	0.60	**0.68**

Table 2. Distribution of 2012 coagulation factor claims payments per utilizing member

	Sum of 2012 Coagulation Factor Payments by Utilizing Member						
Diagnosis	**N Members**	**Mean**	**25th Percentile**	**Median**	**75th Percentile**	**95th Percentile**	**Maximum**
Hemophilia A	257	$231,386	$23,993	$111,992	$248,379	$753,954	$3,141,470
Hemophilia B	71	$144,527	$13,979	$44,535	$223,185	$534,988	$1,126,476
von Willebrand's Disease	55	$29,625	$2,896	$8,762	$24,612	$177,418	$392,443

Results

- Out of 7,895,117 (49.7% male) members continuously enrolled in 2012, 382 had claims for a coagulation factor product and met criteria for classification as Hemo A, Hemo B, or vWD:
  - 257 Hemo A (3.26 per 100,000 members),
  - 71 Hemo B (0.90 per 100,000 members), and
  - 54 vWD (0.68 per 100,000 members).
- **Table 1** shows the prevalence of any coagulation factor treatment by age by diagnosis. Prevalence was significantly higher for each diagnosis among members younger than 25 (6.23, 1.76, and 1.21 per 100,000) compared with members age 25 to 64 (1.68, 0.45, 0.41 per 100,000).
- **Table 2** shows the distribution of coagulation factor claims payments per utilizing member. Total factor cost ranged from $1,139 to $3,141,470 per individual, with mean, median, and ninety-fifth percentiles respectively:
  - Hemo A, $231,386, $111,992, $753,954;
  - Hemo B, $144,527, $44,535, $534,988; and
  - vWD, $29,625, $8,762, $177,418.
- There were 13 members with a claim for a bypassing product: 12/257 (4.7%) Hemo A and 1/71 (1.4%) Hemo B.
- **Figures 1 and 2** show 2012 total factor cost by member plotted by member's age and whether or not treatment included a bypassing product for Hemo A and Hemo B members.
  - Of Hemo A members, 88/257 (34.2%) had factor cost within the predicted range for continuous prophylaxis, 12/257 (4.7%) above and 157/257 (61.1%) below.
  - Of Hemo B, 20/71 (28.2%) had factor cost within the predicted range for continuous prophylaxis, 2/71 (2.8%) above and 49/71 (69.0%) below.

Limitations

- Administrative medical claims have the potential for miscoding resulting in the potential for diagnosis misassignment.
- These results are from a large sample of commercial members younger than 65 years and cannot be extrapolated to other populations. For example, other studies have provided evidence of a strong tendency that older individuals with hemophilia move from employer sponsored to public insurance.[3,4]
- Administrative claims data always lack many pieces of information essential to understand details of coagulation factor therapy. Many paid medical claims imprecisely quantify the number of units administered (or dispensed) to the member. Claims that are for products supplied to member for self-administration (or administration by a family member) do not necessarily indicate that the product was used. This study attempted to estimate the approximate proportions of hemophilia members who were receiving continuous prophylaxis by combining a number of assumptions.

Conclusions

- Among the 7.9 million commercially insured members continuously enrolled in 2012, 382 members (5 per 100,000) had a factor claim and a hemophilia or vWD diagnosis. Individual costs for clotting factor treatments ranged substantially, from $1,139 to $3,141,470 annually, with 13 members having a bypassing product claim and seven of the 13 members having factors costs greater than $1 million. Use of a bypassing claim is a strong signal for the potential to have factor costs greater than $1 million.
- Comparing the actual member hemophilia factor cost to the national age derived weight estimated factor needs, assuming continuous prophylaxis and allowing for substantial weigh variance, still resulted in 62.8 percent of the time overestimating of the annual cost due to the lack of information in the claims about the actual weight, severity of factor deficiency, inhibitor titer, treatment protocol, and member adherence.
- Claims data variables associated with cost variation were diagnosis, age, and use of bypassing products. To better manage factor use, factor products should be billed through the pharmacy benefit and provided by Specialty pharmacies where body weight, severity of factor deficiency, inhibitor titer, treatment protocol, and member adherence information will be used to ensure optimal care.

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