UDC
The Universal Data Collection Program

Report on the Universal Data Collection Program

January 2014

Includes data collected from January 2005 through December 2009

National Center on Birth Defects and Developmental Disabilities
Division of Blood Disorders

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CONTENTS

Summary

The two most common congenital bleeding disorders are von Willebrand disease (VWD) and hemophilia. VWD is caused by the defective synthesis or function of a protein, von Willebrand factor, that is necessary for normal blood clotting. VWD occurs with approximately equal frequency in men and women. Although the prevalence of this disease is not precisely known, it is estimated that up to one percent of the population are affected. There are different types and severity of VWD. Symptoms include heavy or prolonged menstrual bleeding, easy bruising, frequent or prolonged nosebleeds, and prolonged bleeding following surgery, dental work, childbirth or injury.

Hemophilia is caused by a defect in the gene located on the X chromosome that contains the genetic code for one of the clotting factor proteins necessary for normal blood clotting. A deficiency of factor VIII is referred to as hemophilia A or "classic" hemophilia. In contrast, a deficiency of factor IX characterizes hemophilia B, also known as Christmas disease. In females, the presence of the defect on only one of the two X chromosomes results in a carrier state; in some instances female carriers can also experience bleeding symptoms and complications. When males have the defect on their only X chromosome, they have the disease. Thus, almost all of the people with hemophilia disease in the United States are male.

People with severe hemophilia can experience serious bleeding into tissues, muscles, joints, and internal organs, often without any obvious trauma. Repeated bleeding into joints without adequate treatment, results in crippling chronic joint disease, one of the severe complications of bleeding disorders. In the mid-1970s, treatment for hemophilia was improved through the use of clotting factor concentrates, products made from the plasma of donated blood. However, because blood donations from thousands of donors are pooled together to make these products, many people with bleeding disorders were infected with hepatitis B and C viruses and with human immunodeficiency virus (HIV), the virus that causes AIDS, before the risk of disease transmission in blood products was recognized and prevention measures taken.

In 1975, Congress initiated federal funding to specialized hemophilia treatment centers (HTCs) to provide comprehensive care to people with bleeding disorders. In 1983, the Centers for Disease Control and Prevention (CDC) developed programs for people with hemophilia that began with risk-reduction efforts aimed at preventing secondary infection of family members with HIV.

In 1991, CDC received a request from the National Hemophilia Foundation to expand their collaborative activities within the bleeding disorders community. Meetings with patients and hemophilia care providers were held during 1992 to determine the areas of highest priority. Based on recommendations from these constituents, Congress requested that CDC develop programs focused on reducing the human suffering and financial burden of bleeding disorders by focusing national emphasis on prevention and early intervention. The issues of greatest concern identified by the bleeding disorders community were: (1) the safety of the blood supply from infectious diseases and (2) the prevention of joint disease.

In response, CDC developed the Universal Data Collection Program (UDC). The purpose of UDC was two-fold: (1) to establish a sensitive blood safety monitoring system among people with bleeding disorders and (2) to collect a uniform set of clinical outcomes information that can be used to monitor the occurrence of and potential risk factors for infectious diseases and joint complications.

The UDC surveillance project was active from 1998 through September 2011. People with bleeding disorders were enrolled in UDC by care providers in each of the nation's approximately 140 federally funded HTCs. A uniform set of clinical data and blood specimens (plasma or serum) were collected by HTC staff each year during each participant's annual comprehensive clinic visit. A portion of each specimen was used to perform free screening tests for hepatitis A, B, and C viruses and for HIV. The remainder of the specimen was stored for use as needed in future blood safety investigations.

Information about eligibility requirements, enrollment procedures, and data collection can be found in the Technical Notes of this report. Participating HTCs are listed by region in the Acknowledgements. A regional map is also included at the end of this report.

The purpose of this surveillance report is to disseminate the information collected by UDC to care providers, public health workers, community advocates, health educators and planners, and patients in the bleeding disorders community. The report contains information about the demographic characteristics of the participants, their blood and factor product use, and the occurrence and treatment of joint and infectious diseases.

We hope that this information will prove useful to those involved in efforts to reduce or prevent the complications of these conditions.

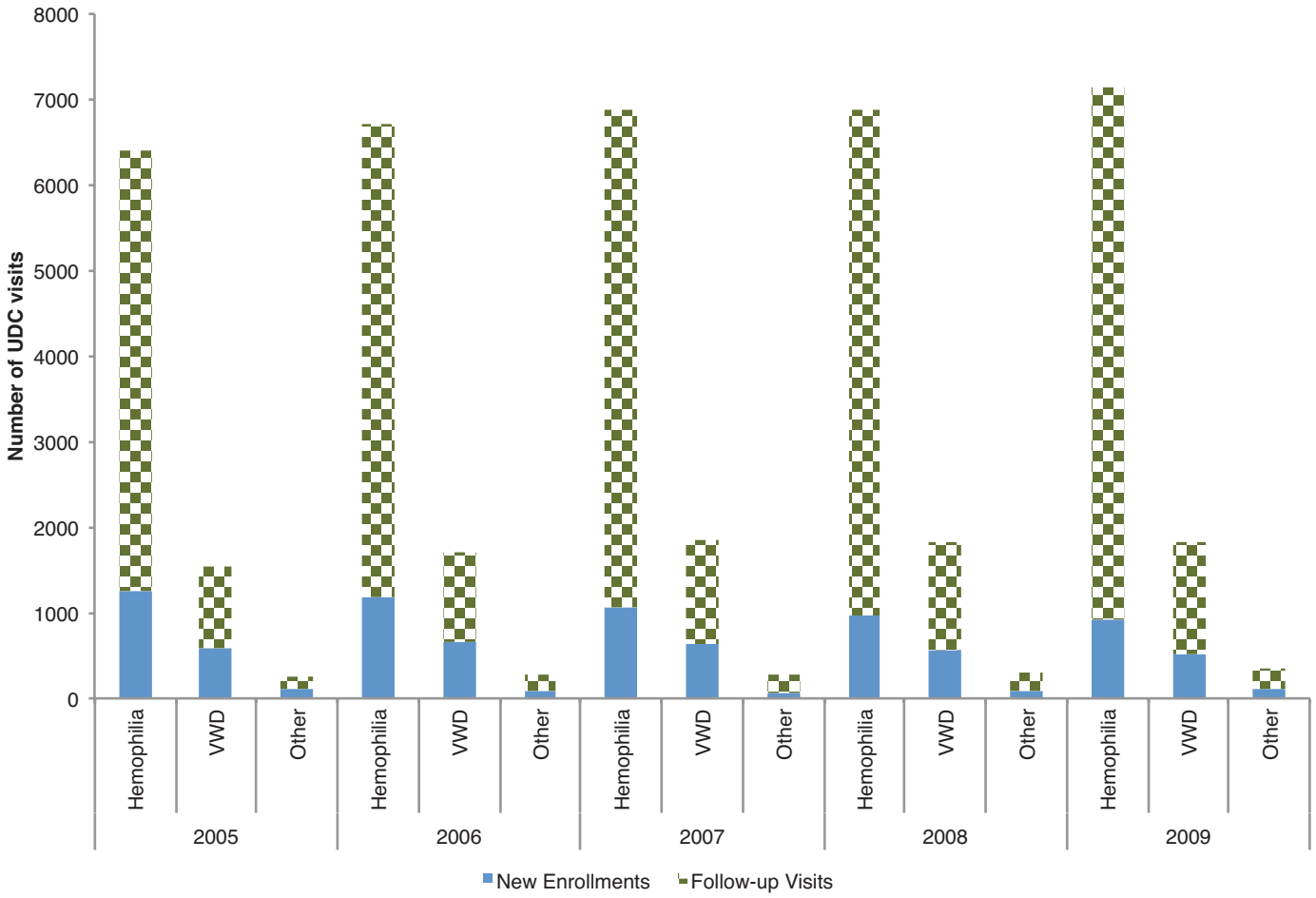
The proper interpretation and appropriate use of surveillance data require an understanding of how the data are collected, reported, and analyzed. Therefore, readers of this report are encouraged to review the Technical Notes that begin on page 21.

Report Highlights

This surveillance report focuses on UDC data collected during years 2005 through 2009. A total of 44,239 visits were made during the five-year interval. Of these, 8,816 were new enrollments and the rest were follow-up visits. People receiving care at HTCs were encouraged to participate in UDC at each visit. When a person was approached to participate in UDC but declined, a record was made. Given the nature of these records it was not possible to make an exact estimation of the refusal rate; however, the maximum and minimum refusal rates are estimated to be 25.3% and 6.3% respectively (please see technical notes).

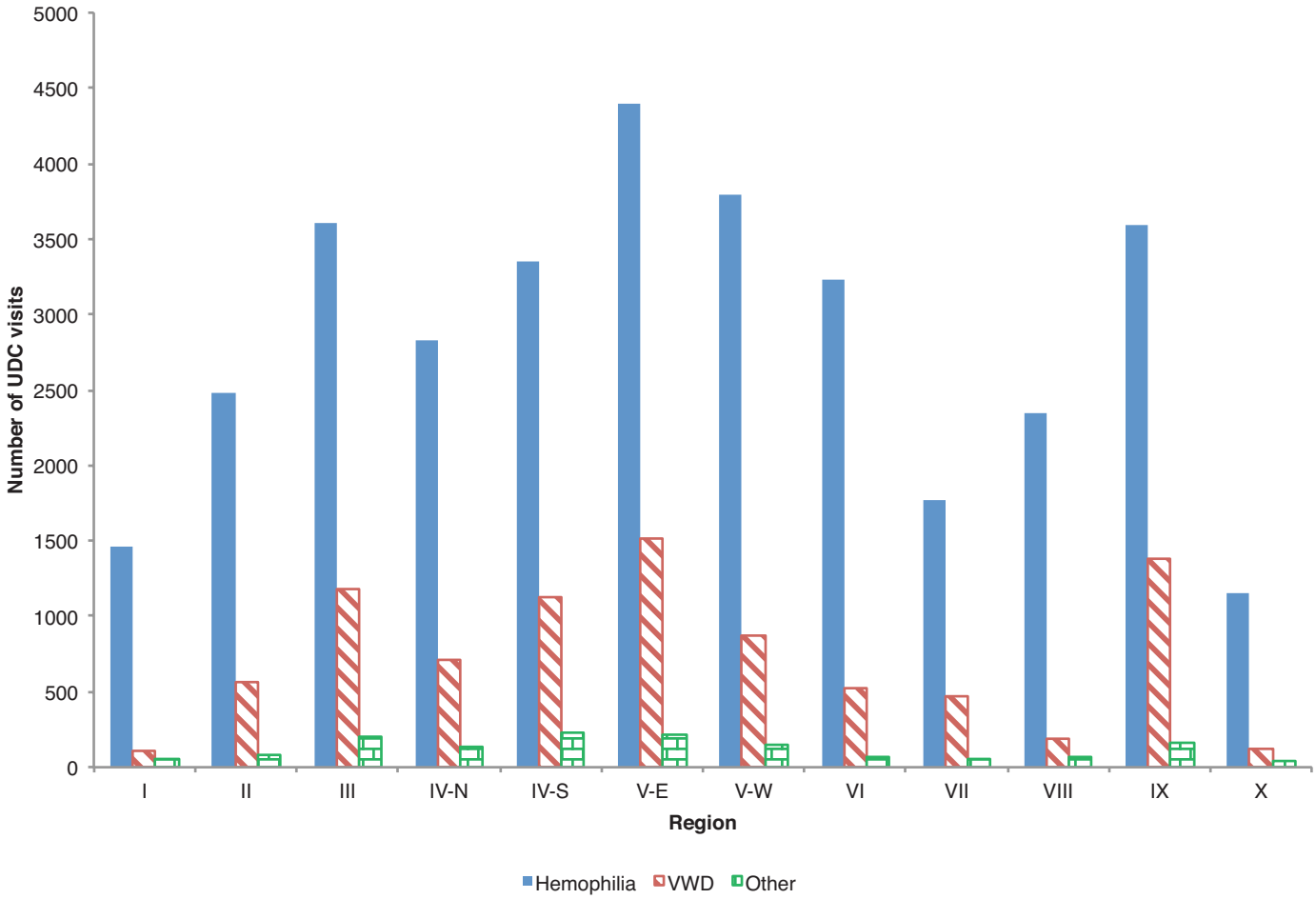
Figures and Tables

Figure 1: New enrollments and follow-up visits in UDC, by diagnosis and year of visit, from January 2005 through December 2009.



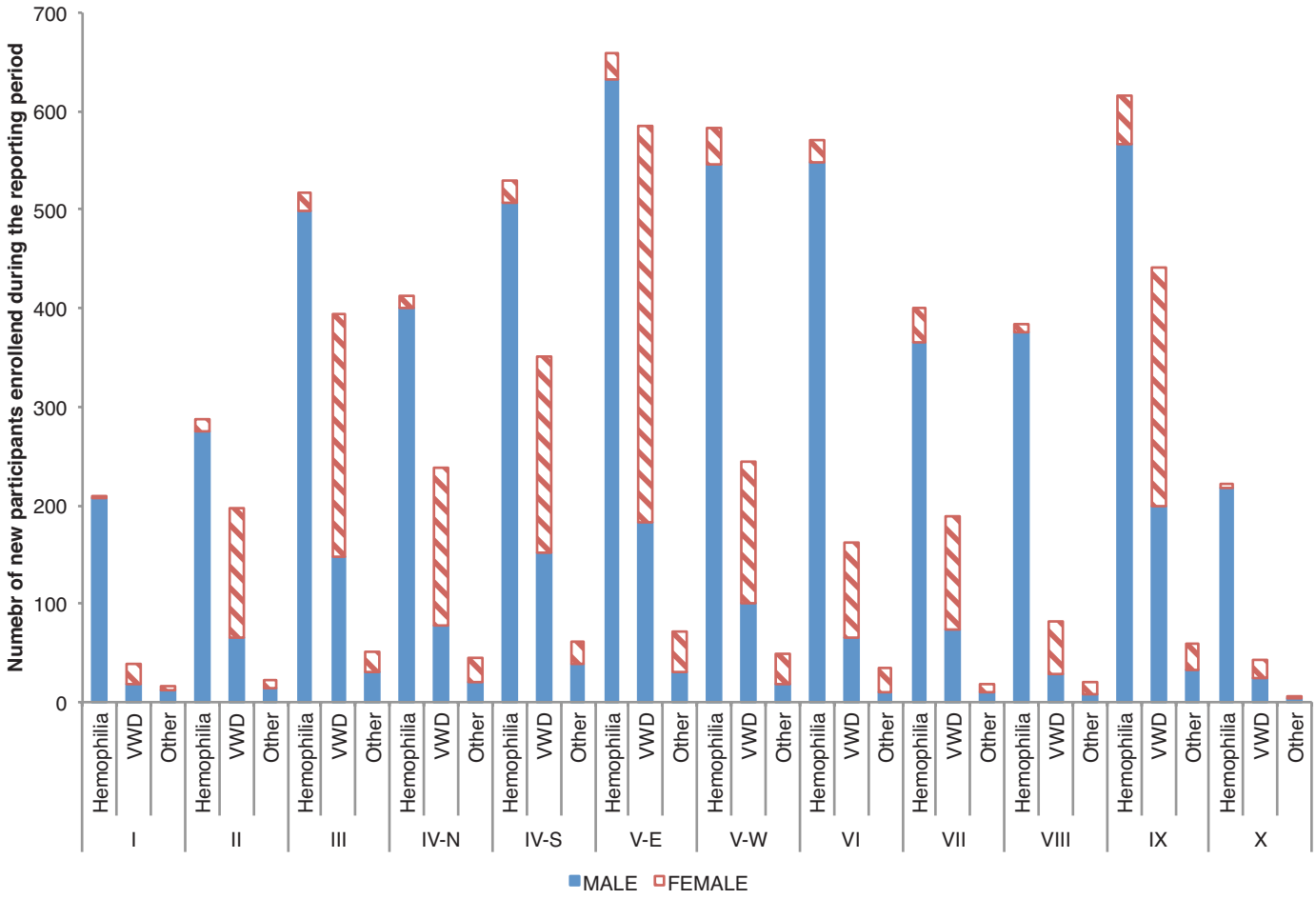
The total number of UDC visits made during the reporting period is shown in figure 1, stratified by diagnosis and the year of the visit. All visits made during the reporting period are shown, including new enrollment visits as well as follow-up visits by all participants irrespective of their time of enrollment. The solid portion of the stacked bars represents new enrollments and the hatched portion represents follow-up visits.

Figure 2: Total UDC visits by region and diagnosis, 2005-2009.



The total number of visits made by participants during the reporting period (2005-2009), by region and diagnosis are shown in figure 2. All visits made during the stated period are presented including new enrollments and follow-up visits by all participants irrespective of their time of enrollment. The largest number of visits was made by people with hemophilia, followed by people with VWD then other bleeding disorders. This pattern is consistent across all regions.

Figure 3A: Total number of new enrollments in UDC 2005-2009, by region, diagnosis and sex of participants.



The distribution of the total number of new UDC participants enrolled during the reporting period (2005-2009) is shown in figure 3A. This distribution is shown stratified by region, diagnosis and sex of the participants. The lower (solid) portion of each bar represents males, whereas the top (hatched) portion represents females. The graph highlights the expected preponderance of males among participants with hemophilia. An overall preponderance of females is seen among participants with VWD.

Figure 3B: Total enrollments in the UDC 2005-2009, by region, diagnosis and age category (at enrollment) of participants.

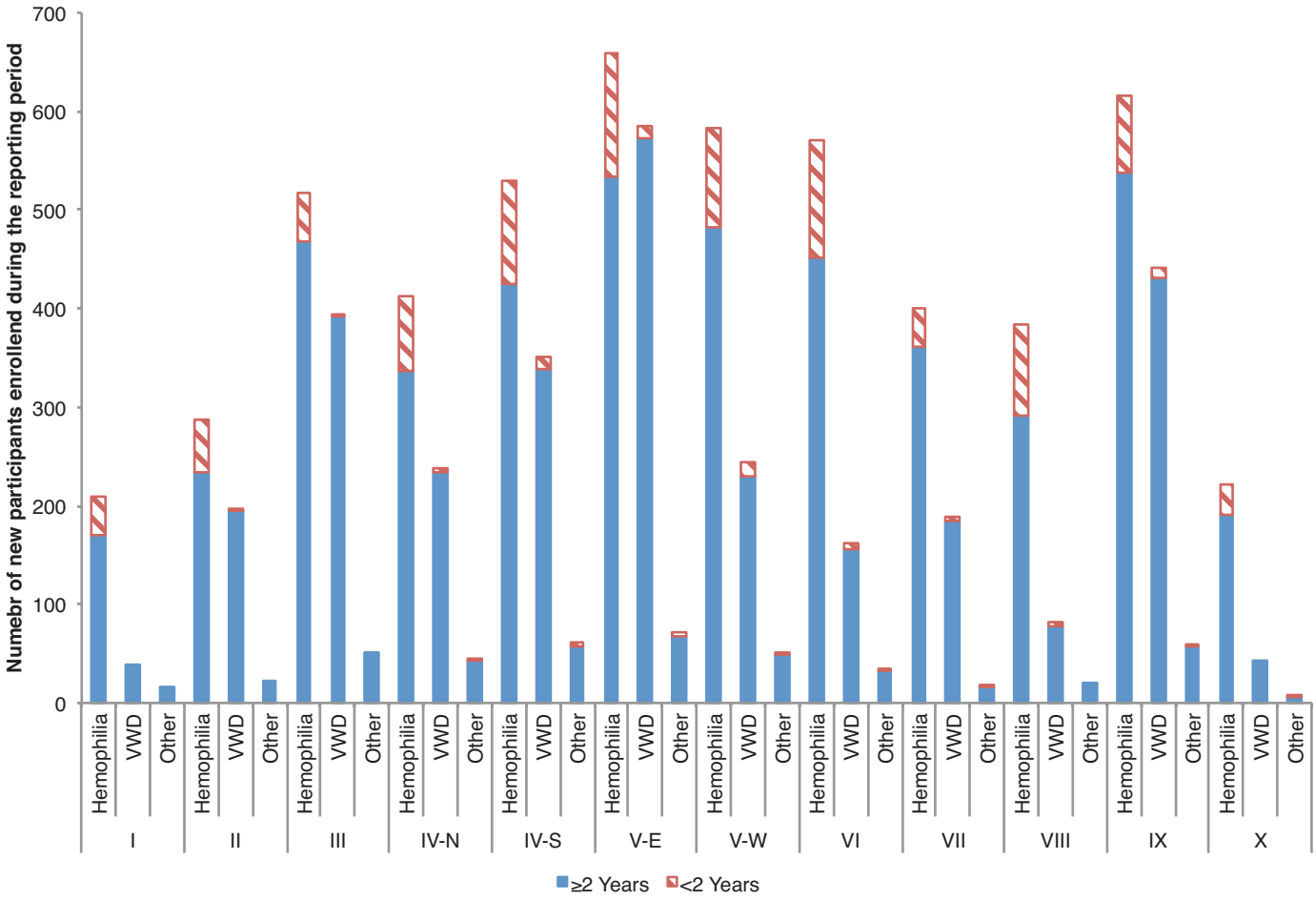


Figure 3B is similar to the previous figure in that it shows the distribution of the total number new UDC participants enrolled during the reporting period (2005-2009). This distribution is shown stratified by region, diagnosis and age category of the participants. The lower (solid) portion of each bar represents participants who were two years old or older at the time of enrollment, whereas the top (hatched) portion represents participants who were younger than two years old at the time of enrollment.

Table 1: Distribution of age and race, by diagnosis and sex, of UDC participants, 2005-2009.

	Hemophilia		VWD		Other[1]	
	Female n (%)	Male n(%)	Female n(%)	Male n(%)	Female n(%)	Male n(%)
Age at follow-up						
<2 years	7 (1.73)	447 (3.41)	23 (0.81)	27 (1.46)	##[2]	9 (2.33)
2-10	63 (15.59)	3027 (23.08)	485 (17.05)	625 (33.69)	61 (17.89)	114 (29.46)
11-20	98 (24.26)	3821 (29.13)	1040 (36.56)	722 (38.92)	99 (29.03)	143 (36.95)
21-40	129 (31.93)	3435 (26.19)	675 (23.73)	232 (12.51)	98 (28.74)	56 (14.47)
41-60	83 (20.54)	1858 (14.16)	489 (17.19)	179 (9.65)	61 (17.89)	38 (9.82)
>60	24 (5.94)	530 (4.04)	133 (4.67)	70 (3.77)	##[2]	27 (6.98)
Race						
White (non-Hispanic)	287 (71.04)	8853 (67.49)	2197 (77.22)	1318 (71.05)	217 (63.64)	230 (59.43)
White (Hispanic)	47 (11.63)	1683 (12.83)	282 (9.91)	232 (12.51)	52 (15.25)	57 (14.73)
Black (non-Hispanic)	20 (4.95)	1570 (11.97)	196 (6.89)	149 (8.03)	42 (12.32)	57 (14.73)
Black (Hispanic)	##[2]	70 (0.53)	9 (0.32)	10 (0.54)	0 (0)	##[2]
Asian/pacific islander	12 (2.97)	358 (2.73)	74 (2.6)	50 (2.7)	7 (2.05)	11 (2.84)
Native American	21 (5.2)	115 (0.88)	8 (0.28)	11 (0.59)	8 (2.35)	##[2]
Other	##[2]	469 (3.58)	79 (2.78)	85 (4.58)	15 (4.4)	29 (7.49)

Table 1 shows the distribution of race/ethnicity and age (years) as of the participants' last contribution to the UDC data. For most participants this corresponded to a follow-up visit made by the end of 2009, but for some participants this was their first, or enrollment, visit. The table is stratified by diagnosis and the sex of the participants. As expected the sex distribution favors males predominantly among participants with hemophilia, and slightly favors females among participants with VWD. In all strata of diagnosis and sex, non-Hispanic White participants form the majority.

1 This category includes participants with coagulation protein disorders other than hemophilia and VWD, hemophilia carriers without type (A or B) specification and participants with more than one diagnosis.

2 Numbers not displayed to protect participants' confidentiality

Table 2: Distribution of age and race/ethnicity, by hemophilia severity and VWD sub-type, of UDC participants, 2005-2009, restricted to participants with known hemophilia type and severity.

	Hemophilia			Von Willebrand Disease		
	Mild n(%)	Moderate n(%)	Severe n(%)	Type 1 n(%)	Type 2 n(%)	Type 3 n(%)
Age at follow-up						
<2 years	105 (3.03)	89 (2.8)	260 (3.81)	30 (0.87)	10 (2.18)	##[3]
2-10	682 (19.66)	738 (23.24)	1662 (24.38)	832 (24.05)	99 (21.57)	48 (18.53)
11-20	1031 (29.72)	937 (29.5)	1937 (28.41)	1380 (39.88)	132 (28.76)	78 (30.12)
21-40	785 (22.63)	763 (24.02)	2000 (29.33)	634 (18.32)	95 (20.7)	74 (28.57)
41-60	616 (17.76)	487 (15.33)	823 (12.07)	462 (13.35)	90 (19.61)	44 (16.99)
>60	250 (7.21)	162 (5.1)	136 (1.99)	122 (3.53)	33 (7.19)	##[3]
Race						
White (Non-Hispanic)	2544 (73.34)	2298 (72.36)	4252 (62.36)	2599 (75.12)	350 (76.25)	201 (77.61)
White (Hispanic)	470 (13.55)	346 (10.89)	909 (13.33)	381 (11.01)	52 (11.33)	18 (6.95)
Black (Non-Hispanic)	229 (6.6)	307 (9.67)	1049 (15.39)	251 (7.25)	32 (6.97)	16 (6.18)
Black (Hispanic)	13 (0.37)	18 (0.57)	43 (0.63)	16 (0.46)	0 (0)	##[3]
Asian/Pacific islander	59 (1.7)	63 (1.98)	247 (3.62)	82 (2.37)	11 (2.4)	9 (3.47)
Native American	51 (1.47)	23 (0.72)	62 (0.91)	15 (0.43)	0 (0)	##[3]
Other	103 (2.97)	121 (3.81)	256 (3.75)	116 (3.35)	14 (3.05)	11 (4.25)

Table 2, shows the distribution of race/ethnicity and age (years) as of the participants' last contribution to the UDC data. For most participants this corresponded to a follow-up visit made by the end of 2009, but for some participants this was their first, or enrollment, visit. Table is stratified by the diagnosis, hemophilia or VWD only, and within the diagnosis by severity (hemophilia) or type (VWD). The majority of participants were between the ages of 11 and 40 years irrespective of diagnosis or severity/type. About half of all the people with hemophilia had the severe form of the disease.

3 Numbers not displayed to protect participants' confidentiality.

Table 3: Primary Insurance type at the last visit, by diagnosis and age group, of UDC participants, 2005-2009.

	Hemophilia		VWD		Other[4]	
	Age < 18 Years n (%)	Age ≥ 18 Years n (%)	Age < 18 Years n (%)	Age ≥ 18 Years n (%)	Age < 18 Years n (%)	Age ≥ 18 Years n (%)
Commercial insurance	3201 (52.76)	3812 (54.20)	1388 (59.75)	1402 (66.16)	191 (56.18)	183 (52.74)
Medicaid	2254 (37.15)	1117 (15.88)	733 (31.55)	253 (11.94)	115 (33.82)	61 (17.58)
Medicare	58 (0.96)	1128 (16.04)	18 (0.77)	215 (10.15)	5 (1.47)	53 (15.27)
Other	64 (1.05)	35 (0.50)	##[5]	##[5]	##[5]	8 (2.31)
State Programs	235 (3.87)	458 (6.51)	80 (3.44)	80 (3.78)	11 (3.24)	20 (5.76)
Tricare/Military	108 (1.78)	30 (0.43)	40 (1.72)	20 (0.94)	5 (1.47)	6 (1.73)
Uninsured	129 (2.13)	443 (6.30)	37 (1.59)	137 (6.47)	9 (2.65)	16 (4.61)
Unknown	18 (0.3)	10 (0.14)	##[5]	##[5]	##[5]	0 (0.0)

Table 3 shows the type of medical insurance carried by the participants as recorded at the time of the last UDC visit, stratified by diagnosis and age category. Across all diagnoses and age groups the majority of participants reported carrying commercial insurance.

4 This category includes participants with coagulation protein disorders other than hemophilia and VWD, hemophilia carriers without type (A or B) specification and participants with more than one diagnosis.

5 Numbers not displayed to protect participants' confidentiality.

Table 4A: Mean (standard deviation), of number of bleeds at select sites over the six months before the last visit of UDC participants with hemophilia, two years old or older, by severity and treatment type, 2005-2009, restricted to participants with known hemophilia type and severity.

	On prophylaxis (continuous or intermittent)				Episodic treatment (No prophylaxis)			
	Mild n=96	Moderate n=628	Severe n=3682	Unknown n=5	Mild n=3135	Moderate n=2379	Severe n=2736	Unknown n=13
Bleeding Site								
Joint	1.5 (5.4)	1.1 (4.0)	1.1 (4.1)	0.4 (0.9)	0.2 (1.2)	0.7 (2.9)	2.7 (7.8)	2.7 (7.0)
Muscle	0.3 (0.9)	0.3 (1.3)	0.3 (1.6)	0 (0)	0.1 (0.5)	0.2 (1.5)	0.6 (2.2)	0.5 (1.5)
Other	0.4 (1.4)	0.3 (3.0)	0.3 (1.6)	1 (1.2)	0.2 (1.3)	0.4 (2.6)	0.5 (2.3)	2.0 (6.6)
All sites								
Mean (SD)	2.2 (5.8)	1.7 (5.8)	1.7 (5.5)	1.4 (1.5)	0.5 (2.0)	1.3 (4.6)	3.6 (9.6)	5.2 (11.8)
Median	0	0	0	1	0	0	0	0

At each annual UDC visit, the number of bleeds experienced by the participant in the prior six months and the sites of those bleeds was recorded through a combination of medical records abstraction and patient/parent interview. Bleeding sites recorded varied depending on whether, at the time of the visit, the participant was at least 2 years old or younger.

Table 4A, restricted to people with hemophilia who were at least two years old at their last UDC visit, presents the mean and standard deviation of the number of bleeds recorded at their last UDC visit. This table is stratified by whether the participant was recorded to be receiving prophylactic factor infusion therapy or on-demand therapy with further stratification by hemophilia severity.

Table 4B: Mean (standard deviation), of number of bleeds at select sites, since birth[6] or the previous annual visit of UDC participants with hemophilia younger than two years of age by severity and treatment type, 2005-2009, restricted to participants with known hemophilia type and severity.

	On prophylaxis (continuous or Intermittent)		Episodic treatment (No prophylaxis)		
	Moderate n=9	Severe n=51	Mild n=91	Moderate n=64	Severe n=171
Bleeding					
Intracranial	0.13 (0.35)	0.26 (0.49)	0.04 (0.24)	0.02 (0.13)	0.05 (0.21)
Circumcision	0 (0.0)	1.3 (0.95)	1.2 (0.45)	1 (0.0)	1 (0.0)
Oral/nasal	1.67 (0.58)	2.1 (1.2)	1.81 (2.29)	1.82 (1.24)	1.45 (0.83)
Venipuncture/heel stick/ surgical site	1 (0.0)	1.2 (0.42)	1 (0.0)	1.67 (1.15)	1.43 (0.73)
Soft tissue hematoma	3.6 (2.41)	4.85 (5.61)	1.29 (0.49)	2.88 (4.16)	3.99 (5.69)
Intramuscular hematoma	1 (0.0)	1.13 (0.83)	1 (0.0)	1.5 (0.71)	1.5 (0.99)
Umbilicus	0 (0.0)	0 (0.0)	1 (0.0)	0 (0.0)	0 (0.0)
Joint	1 (0.0)	1.73 (0.88)	1 (0.0)	1.6 (1.34)	1.5 (0.83)
Gastrointestinal tract	0 (0.0)	1 (0.0)	1 (0.0)	0 (0.0)	3 (2.83)
Genitourinary/renal	0 (0.0)	2 (1.41)	0 (0.0)	0 (0.0)	1 (0.0)
Pulmonary	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
All sites					
Mean (SD)	3.33 (3.43)	3.88 (5.22)	0.81 (1.96)	1.65 (3.14)	3.16 (5.08)
Median	2	2	0	1	2

Table 4B is restricted to participants with hemophilia who were younger than 2 years of age at the time of their last visit. Like the previous table, this table presents the mean and standard deviation of the number of bleeds recorded at their last UDC visit. This table is stratified by whether the participant was recorded to be receiving prophylactic factor infusion therapy or on-demand therapy with further stratification by hemophilia severity. In addition to the mean and standard deviation of the number of reported bleeds by the sites of interest, the overall (spanning all bleeding sites) mean and standard deviations and medians are also reported. There were too few participants with mild hemophilia on prophylaxis to report.

6 Since birth, if it was participant's only annual visit before reaching 2 years of age.

Table 5A: Mean (standard deviation), of number of bleeds at select sites over the six months before the last visit of UDC participants with von Willebrand disease two years old or older by type, 2005-2009.

	Type 1 n=3232	Type 2 n=432	Type 3 n=249	Unknown n=484
Bleeding Site				
Joint	0.03 (0.27)	0.04 (0.44)	0.54 (2.0)	0.17 (1.25)
Muscle	0.01 (0.22)	0.01 (0.1)	0.15 (0.86)	0.06 (0.52)
Other	0.95 (7.13)	1.49 (7.77)	2.02 (9.94)	0.64 (3.19)
All sites				
Mean (SD)	0.99 (7.19)	1.54 (7.84)	2.71 (10.18)	0.87 (3.65)
Median	0.0	0.0	0.0	0.0

Table 5A presents the mean and standard deviation of the number of bleeds recorded for people with VWD as reported at the last UDC visit, stratified by VWD type. Table 5A is restricted to participants at least 2 years old. In addition to the mean and standard deviation of the number of reported bleeds by the sites of interest, the overall (spanning all bleeding sites) mean and standard deviations and median are also reported.

Table 5B: Mean (standard deviation), of number of bleeds at select sites, since birth[7] or the previous annual visit of UDC participants with von Willebrand Disease younger than two years of age by type, 2005-2009.

	Type 1 n=28	Type 2 n=6	Unknown n=8
Bleeding Site			
Intracranial	0.15 (0.61)	0 (0.0)	0 (0.0)
Circumcision	1 (0.0)	0 (0.0)	0 (0.0)
Oral/nasal	6.89 (10.71)	1.33 (0.58)	2 (0.0)
Venipuncture/heel stick/ surgical site	0 (0.0)	0 (0.0)	1.5 (0.71)
Soft tissue hematoma	1.67 (0.58)	3 (0.0)	25 (0.0)
Intramuscular hematoma	0 (0.0)	0 (0.0)	0 (0.0)
Umbilicus	0 (0.0)	0 (0.0)	0 (0.0)
Joint	0 (0.0)	0 (0.0)	0 (0.0)
Gastrointestinal tract	0 (0.0)	0 (0.0)	0 (0.0)
Genitourinary/renal	0 (0.0)	0 (0.0)	0 (0.0)
Pulmonary	0 (0.0)	0 (0.0)	0 (0.0)
All sites			
Mean (SD)	2.74 (6.99)	1.17 (1.6)	4 (8.57)
Median	0	0.5	1

Table 5B presents the mean and standard deviation of the number of bleeds recorded for people with VWD as reported at the last UDC visit, stratified by VWD type. Table 5B is restricted to participants who were younger than 2 years at the time of their last visit. In addition to the mean and standard deviation of the number of reported bleeds by the sites of interest, the overall (spanning all bleeding sites) mean and standard deviations and median are also reported. There were too few participants with type 3 VWD younger than two years of age to report.

7 Since birth, if it was participant's only annual visit before reaching 2 years of age.

Table 6: Liver disease risk factors and signs and symptoms; treatment for chronic viral hepatitis and central venous access device used, by diagnosis, of UDC participants two years old or older at last visit, 2005-2009.

	Hemophilia n =12711	VWD n = 4256	Other[8] n = 717
Risk factors for liver disease	n(%)	n(%)	n(%)
Past/present hepatitis B infection[9]	1678 (13.2)	142 (3.23)	30 (4.18)
Past/present hepatitis C infection[9]	3735 (29.38)	205 (4.66)	49 (6.83)
History of alcohol abuse	368 (2.9)	21 (0.48)	##[10]
Other	133 (1.05)	18 (0.4)	##[10]
Signs or symptoms of liver disease since previous visit			
Jaundice	21 (0.17)	##[10]	##[10]
Ascites	41 (0.32)	##[10]	##[10]
Varices	40 (0.31)	##[10]	##[10]
Other	56 (0.44)	6 (0.14)	6 (0.8)
Laboratory markers of liver disease since previous visit			
Clinically elevated ALT/AST	904 (7.11)	61 (1.39)	61 (1.39)
Elevated prothrombin time	149 (1.17)	20 (0.45)	20 (0.45)
Therapy for chronic viral hepatitis ever, or since previous visit[11]			
Any therapy	1076 (8.47)	58 (1.32)	17 (2.4)
Successful therapy[12]	501 (46.52)	26 (44.83)	9 (52.9)
Central venous access device (CVAD)			
Used since previous visit	1328 (10.45)	77 (1.75)	33 (4.6)
Associated infection[13]	157 (11.81)	11 (14.29)	0 (0.0)

Table 6 shows the distribution of risk factors for and signs and symptoms of liver disease, treatment for viral hepatitis and use of central venous access devices (CVAD), stratified by diagnosis. Most participants have no risk factors for liver disease, though a larger proportion of participants with hemophilia report the presence of liver disease risk factors than those with VWD or another diagnosis. A greater proportion of all participants regardless of diagnosis reported past or present infection with hepatitis C than any other risk factor. Among people who received therapy for chronic viral hepatitis, successful therapy was reported for about half in each diagnostic category. Among people with hemophilia, central venous access device (CVAD) use was reported for about 10%. A CVAD-associated infection was reported for about 12% of people with hemophilia for whom CVAD use was reported. In people with VWD, CVAD use and CVAD-associated infection reports were about 2% and 15% respectively.

8 This category includes participants with coagulation protein disorders other than hemophilia and VWD, hemophilia carriers without type (A or B) specification and participants with more than one diagnosis.

9 As reported in annual visit data, not from laboratory data.

10 Numbers not displayed to protect participants' confidentiality.

11 Ever, if it was participant's first annual visit

12 Restricted to participants who received any therapy for viral hepatitis.

13 Restricted to participants who used a CVAD.

Table 7: Treatment type at the last visit of UDC participants with hemophilia, by severity, 2005-2009, restricted to participants with known hemophilia type and severity.

	Mild	Moderate	Severe	Unknown
Treatment	n (%)	n (%)	n (%)	n (%)
Episodic care	3219 (96.87)	2431 (78.88)	2775 (41.79)	13 (72.22)
Intermittent prophylaxis	27 (0.81)	137 (4.45)	544 (8.19)	0 (0.0)
Continuous prophylaxis	70 (2.11)	501 (16.26)	3187 (47.99)	5 (27.78)
Immune tolerance therapy	7 (0.21)	11 (0.36)	133 (2.0)	0 (0.0)

Table 7 shows the treatment type for people with hemophilia at the last UDC visit, stratified by severity of hemophilia. People with mild or moderate hemophilia predominantly received episodic care, whereas almost half (47.9%) of those with severe hemophilia received continuous prophylactic treatment (see Technical Notes).

Table 8: Prevalence of inhibitors, categorized as low or high titer[14] among people with hemophilia, by type and severity, UDC participants 2005-2009, restricted to participants with known hemophilia type and severity.

	Hemophilia A (FVIII)				Hemophilia B (FIX)			
	Number	Low titer[15]	High titer[16]	Titer not done	Number	Low titer[15]	High titer[16]	Titer not done
Severity		n(%)	n(%)			n(%)	n(%)	
Mild	2672	26 (0.97)	11 (0.41)	1588	797	11 (1.38)	0 (0.0)	474
Moderate	2056	56 (2.72)	27 (1.31)	762	1120	15 (1.34)	1 (0.09)	595
Severe	5753	293 (5.09)	350 (6.08)	1684	1065	35 (3.29)	30 (2. 82)	376

Table 8 presents the prevalence of an inhibitor to factor treatment product, categorized as low (1-<5 Bethesda units) or high (≥5 Bethesda units) (please see technical notes), in people with hemophilia stratified by hemophilia type (A or B). In both hemophilia types, the highest proportion of high titers is seen among those with severe disease. Almost 39% of participants with hemophilia A and 49% of those with hemophilia B were never tested for inhibitors while participating in UDC ("titer not done"). The highest proportion of individuals tested for inhibitors were those with severe disease.

14 Inhibitor titer category is determined by the highest recorded inhibitor titer during the reporting period.

15 Low titer is defined as reporting a maximum inhibitor titer between 1 and <5 Bethesda units (BU).

16 High titer is defined as reporting a maximum inhibitor titer ≥5 Bethesda units (BU).

Table 9: Treatment products used by UDC participants, by diagnosis, as reported at their last visit, 2005-2009.

	Hemophilia A n=10184	Hemophilia B n=2882	VWD n=4442	Other disorders[17] n=724
Treatment products	n(%)	n(%)	n(%)	n(%)
Recombinant products	7299 (71.50)	1868 (64.61)	26 (0.59)	56 (8.15)
Monoclonal	742 (7.27)	##[18]	##[18]	##[18]
Blood bank products	35 (0.34)	9 (0.31)	33 (0.74)	90 (13.10)
Human FIX	6 (0.06)	391 (13.52)	##[18]	##[18]
Prothrombin complex	20 (0.20)	##[18]	0 (0.0)	28 (4.08)
Activated prothrombin complex	288 (2.82)	17 (0.59)	##[18]	8 (1.16)
Human FVIII	221 (2.16)	9 (0.31)	1113 (25.06)	27 (3.93)
Other factor concentrates	226 (2.21)	40 (1.38)	11 (0.25)	108 (15.72)
Intravenous desmopressin	101 (0.99)	5 (0.17)	369 (8.31)	12 (1.75)
Nasal desmopressin	755 (7.40)	9 (0.31)	1582 (35.61)	37 (5.39)
Topical Amicar	1448 (14.18)	350 (12.11)	1171 (26.36)	123 (17.90)
Other non-plasma or topical	23 (0.23)	8 (0.28)	29 (0.65)	5 (0.73)
None used	1262 (12.36)	596 (20.62)	1583 (35.64)	310 (45.12)

Tables 9 through 11 summarize the use of treatment products, presented in 12 categories.

Table 9 shows the use of treatment products by bleeding disorder diagnosis, with hemophilia further broken down by type. The last row shows the number and percent of people within each diagnostic category who did not use any treatment product during the reporting period.

17 This category includes participants with coagulation protein disorders other than hemophilia and VWD, hemophilia carriers without type (A or B) specification and participants with more than one diagnosis.

18 Numbers not displayed to protect participants' confidentiality.

Table 10: Multiple product usage reported at the last visit among UDC participants with hemophilia, by treatment type, 2005-2009.

	Total using at least one product	Using more than one product
Treatment type	n	n(%)
Episodic care	9705	2722 (28.05)
Intermittent prophylaxis	748	103 (13.77)
Continuous prophylaxis	3871	758 (19.58)

Table 10 is restricted to participants with hemophilia and presents data as reported at their last UDC visit. The numbers of participants using at least one treatment product are presented. The number and percent of participants who received more than one treatment product is also shown. The rows divide the numbers by treatment type recorded at the last UDC visit, i.e., continuous prophylaxis, intermittent prophylaxis or episodic care (see Technical Notes).

Table 11: Multiple product usage reported at the last visit among UDC participants with hemophilia, by severity, 2005-2009, restricted to participants with known hemophilia type and severity.

	Total using at least one product	Using more than one product
Hemophilia severity	n	n(%)
Mild	2241	659 (29.41)
Moderate	2600	547 (21.04)
Severe	6370	1119 (17.57)
Unknown	13	3 (23.08)
All	11224	2328 (20.74)

Table 11 is restricted to people with hemophilia and presents data as reported at their last UDC visit. The numbers of participants using at least one treatment product are presented. The number and percent of participants who received more than one treatment product is also presented. The data are stratified according to the severity of hemophilia, i.e., mild, moderate, severe or unknown.

Technical notes

Eligibility Requirements

To participate in UDC, patients must have received care in a federally funded HTC and met at least one of the following criteria: (1) have a bleeding disorder due to congenital deficiency or acquired inhibitors in which any of the coagulation proteins is missing, reduced, or defective and has a functional level of less than 50 %; or (2) have a diagnosis by a physician of VWD. Individuals specifically excluded from participation in UDC include persons with any of the following: (1) an exclusive diagnosis of a platelet disorder, (2) thrombophilia, or (3) coagulation protein deficiencies due to liver failure. Initially, participation was limited to those 2 years old or older, but in 2003 eligibility was extended to babies and children under the age of 2 years. Data were collected using special "Baby" surveillance forms up to twice per year until age 2 years. No blood specimens were collected from participants younger than 2 years old.

Data Collection

UDC data were collected during a participant's "annual visit", which ideally occurred once each calendar year (January—December), with the interval between visits as close as possible to 12 months. Data were collected according to guidelines and definitions detailed in surveillance manuals provided to HTC staff by CDC through a combination of medical records abstraction and patient interview. Informed consent for participation was obtained. Demographic information and reasons for refusal were obtained using a refusal form for all eligible people who declined participation. To protect patient confidentiality, all data sent to CDC did not contain personal identifying information, but rather used a unique 12-digit code generated by a computer software program supplied to HTCs by CDC.

Eligible participants were enrolled into UDC through a registration form completed by HTC staff; information collected on this form includes patient demographic, diagnostic, and historical information. Month and year of birth are used to calculate age on the last day of the month of the visit. Information on race and ethnicity might be based either on self-report or on observations made by care providers. During the enrollment visit and at subsequent annual visits, clinical information was recorded on a standardized annual visit data collection form. In addition to information about education, employment status and health insurance, data were also collected about the type of treatment (episodic vs. prophylactic), presence and treatment of inhibitors, the number of bleeding episodes experienced (based on infusion logs or patient recall), the type and brand name of all factor concentrates or other treatment products used, whether or not clotting factor was infused at home and other items. Prophylactic treatment and its types were defined in UDC as follows: If the patient received treatment products to prevent bleeding or to prevent re-bleeding, and the duration of treatment was not less than 28 days then the person was said to be on prophylactic treatment. If a person on prophylaxis was recommended to receive treatment products on a regular schedule to prevent any and all bleeding and this therapy was expected to continue indefinitely, the prophylactic treatment was defined as 'continuous'. If the patient received treatment products on a regular schedule for a period of at least 28 days on at least one occasion since the last annual visit and this therapy was not expected to continue for an indefinite period of time, the person was said to be on intermittent prophylaxis. Copies of the complete data collection forms with definitions may be accessed at: http://www.cdc.gov/ncbddd/blooddisorders/udc/udc-hemophilia.html#forms.

Every time a person declined UDC participation, a record was made of the refusal. However, as refusal forms were not labeled with a person-specific identifier, these 'refusals' data represent a count of the number of refusals made by people with bleeding disorders visiting an HTC, and not unique individuals. Therefore, it is not possible to report the exact proportion of people approached who refused to participate. However, to gain some sense of a refusal 'rate' we took two different approaches to estimate conservative (maximum) and liberal (minimum) refusal rates. Total refusals to participate in the UDC during 2005-2009 were 2992, whereas a total of 8816 new participants were enrolled in the same time period. Taking the most conservative approach, and assuming that each recorded refusal represented a unique individual, the maximum refusal rate was calculated to be 2992/(8816+2992) = 25.3%. To estimate the minimum refusal rate, we divided the same numerator of 2992 by the sum of total refusals (2992) and total visits (44293), yielding 2992/(44293+2992) = 6.3%. This approach for estimation of the low end of the refusal rate is justified given that UDC participants had the opportunity to decline participation at each annual UDC visit. The two estimates represent extreme values and the actual refusal rate will be somewhere between the two estimates.

Health insurance was recorded as a 10-category variable with the option to write-in other health insurance information if it did not fit into one of the pre-coded options. In the few events of multiple insurance reports, the primary insurance was determined by an algorithm using the following hierarchy: Medicare, commercial, Medicaid, Tricare, state programs, uninsured, and unknown.

At each visit, the number of joint, muscle and other bleeds experienced by the participant during the six month period prior to the visit was recorded. Reports of bleeds could be obtained either from a log (if the participant maintained one) or from recall in the absence of a log. In the very few instances where a record of bleeds was made in both fields (log and recall), the number reported from the log was used.

Information regarding viral hepatitis and HIV was also collected, including risk factors and clinical signs, symptoms, and laboratory markers of liver disease. Data were also recorded about any therapy for chronic hepatitis; and, among patients with a central venous access device, the occurrence of a device-associated infection.

Data collection forms were sent to CDC where they were entered into a computer database using double-entry software to minimize data entry errors. Data were then screened for omissions, inconsistencies, and unusual values that possibly represented abstraction or data-entry errors. Error reports were generated and faxed to the HTC, where a designated UDC contact used available information to resolve discrepancies and complete missing data items. Beginning in the year 2002, the HTC network, with CDC provided specifications, supported the development and use of a clinical database software tool with the capacity to validate and store the data collected for UDC. HTCs could choose to enter their data into this tool (Labtracker™) for validation and electronic transmission to CDC. A copy of the data was stored at the local facility as backup and for archival purposes. During the 2005-2009 reporting period, data from 28505 (64.4%) HTC visits were sent to CDC via Labtracker™.

Laboratory Testing

During the annual visit, a blood specimen was obtained from each participant age 2 years old or older. Specimens were processed by HTC personnel according to guidelines provided by CDC designed to minimize the effects of storage and shipment on subsequent analyses. Samples were shipped overnight to the CDC Serum Bank where they were aliquoted and stored. A portion of each specimen was sent to the Division of Blood Disorders laboratory at CDC. The remainder of the specimen was stored in the CDC Serum Bank for future blood safety investigations, as needed.

Testing for hepatitis A, B, and C viruses followed algorithms designed to determine with the highest probability the patient's status with regard to exposure to or infection with these viruses. Information provided by HTC staff on a Laboratory Form, including vaccination history, was used by personnel at the testing laboratory to provide a detailed interpretation of the test results.

Tabulation and Presentation of Data

Data in this report are provisional. Missing data prevents the totals for some tables from adding up to the total sample size. Counts of 1-4 within a table cell are not presented in order to protect the confidentiality of participants; in some cases this required the suppression of data in an additional cell within that column to prevent mathematical derivation of the count. The data represented were collected during 2005-2009 from an on-going surveillance project. The current report is a follow-up to the first five year report published in July 2005, which reported on data collected from 1998-2004. A future comprehensive report will include expanded data tables to cover the entire surveillance period and will provide the results of more detailed analyses of available data.

Figure or table specific comments:

Figure 1: As an annual visit data form was required to be completed at enrollment, the participant's first annual visit form was not counted as a follow-up visit, unless – as in a few instances – six months or more lapsed between completion of the registration form and the initial annual visit form.

Table 1: The 'other bleeding disorders' category includes participants coded as "hemophilia carrier" without specification of the hemophilia type (A or B). People with multiple diagnoses were also included in this category.

Table 6: Hepatitis C and B status are from annual visit data and are not based on laboratory testing performed for the UDC project.

Table 8: Categorization of inhibitors as low or high titer is based on the highest inhibitor titer recorded during the reporting period.

Acknowledgements

Note: Since the collection of the data represented in this report, the federal regions for hemophilia care have been reorganized by the Health Resources and Services Administration. The acknowledgements made here, as well as the regional map, reflect the regional structure and project organization at the time the data was collected.

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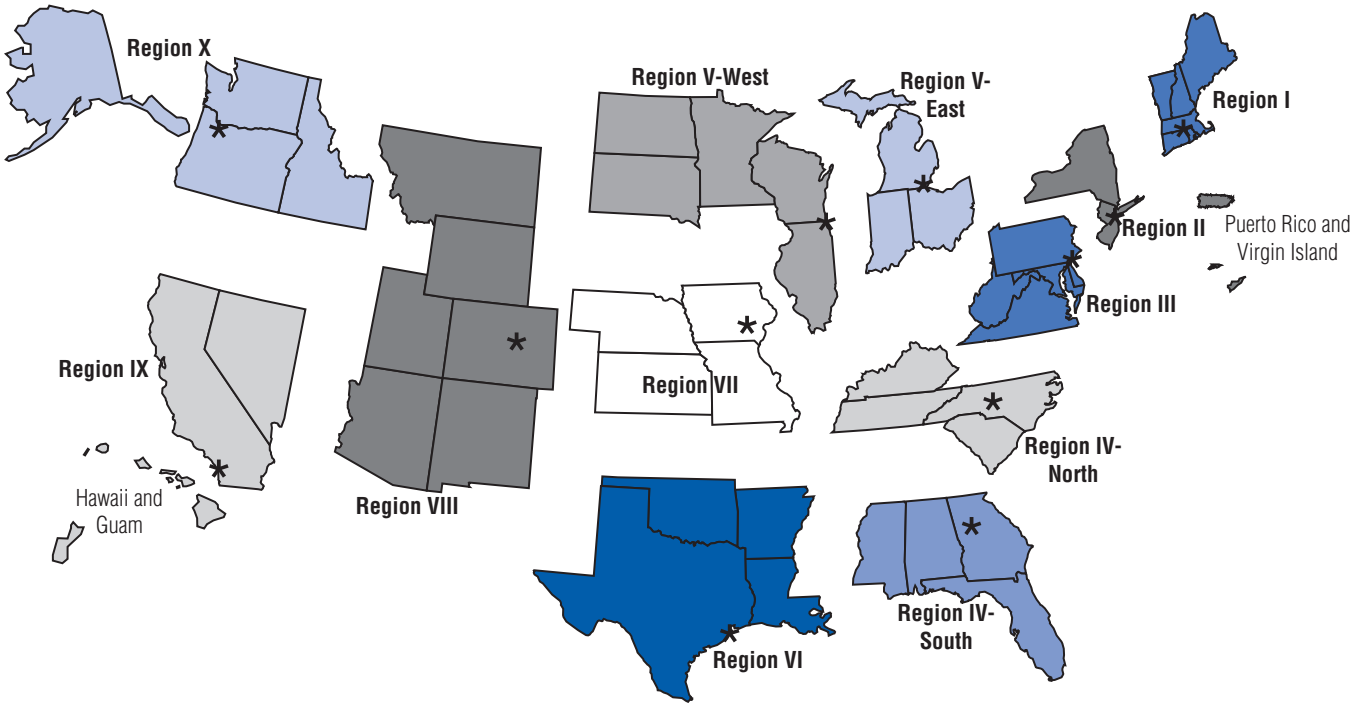

* Locations of regional core center