Public Health Surveillance of People Not Receiving Care at US Federally-funded Hemophilia Treatment Centers: Treatment, Sources, and Utilization of Healthcare in the CHOICE Project

Authors: Wendy E. Owens[1], Meredith A. Oakley[2], Binh C. Le[2] and Vanessa R. Byams[2]

Affiliations:

1 Hemophilia Federation of America, Washington, DC

2 Division of Blood Disorders, National Center for Birth Defects and Developmental Disabilities, Centers for Disease Control and Prevention, Atlanta, GA

Introduction

Congressional funding of hemophilia treatment centers (HTC) through the Health Resources and Services Administration (HRSA) began in 1975 after studies demonstrated that care in these centers reduced complications in persons with hemophilia (PWH)[1]. Population-based surveillance of hemophilia conducted by The US Centers for Disease Control and Prevention (CDC) from 1993-1995 (the Hemophilia Surveillance System or HSS) demonstrated that (1) 67% of PWH visited a federally-funded HTC at least once during the three-year period of ascertainment and (2) the risk of death and bleed-related hospitalizations were each decreased by 40% in persons seen at HTCs compared to those receiving care elsewhere[2, 3, 4]. More recent estimates suggest that 70% of the estimated 20,000 PWH in the US are seen at HTCs[5]. These estimates suggest that perhaps 6,000 PWH, and an unknown number of persons with other genetic bleeding disorders, receive care elsewhere (non-HTC PWBD). Data on non-HTC PWBD are not collected on a regular basis nor maintained in a central database. Consequently, little is known about their characteristics, treatment, or care. CDC partnered with a national non-profit, consumer-focused organization, Hemophilia Federation of America (HFA), on the CHOICE (Community Having Opportunity to Influence Care Equity) Project to collect information on non-HTC PWBD in order to better estimate disease burden and inform planning, decision making, and programming. The objective of this analysis is to describe the treatment, sources, and patterns of care for non-HTC PWBD.

Methods

HFA and CDC collaborated on the development of the CHOICE survey questions with the input from members of the bleeding disorders community. From 04/2013-07/2015, HFA recruited US PWBD >/= 18 years old (adults) and caregivers of children with bleeding disorders for CHOICE through email, social media, print, and word-of-mouth to take an approximately 20-minute survey in English or Spanish, online or on paper. Non-HTC PWBD were solicited specifically but others were not excluded from participation. A series of questions was included in the CHOICE survey to help establish objectively whether a respondent received care at an HTC. Participants' status as non-HTC PWBD was assigned using an algorithm based on their response to these questions. Surveys that were missing data for key variables (respondent status [self vs. caregiver], sex and diagnosis), had an ineligible diagnosis, were non-US residents, or that appeared to be duplicates were excluded from analysis. For this analysis, survey elements included demographics, diagnosis, treatment regimen and treatment products used, and health services utilization (usual source of care, frequency of care, barriers to regular care, delay of care, hospital emergency room (ER) utilization, and hospitalizations within the past 12 months). SAS version 9.3 software[6] was used to compute frequencies for all elements, as well as the Pearson chi-square or Fisher's exact test to check for the association of sex, age (child vs adult), ethnicity, and race (white vs non-white) with certain outcome variables.

Results

Of the 719 eligible surveys, 681 (95%) were completed in English and 38 (5%) were completed in Spanish; 698 (97%) were completed electronically and 21 (3%) on paper. Twenty-two percent of participants (161) were identified as non-HTC PWBD. Fifty-two percent of non-HTC PWBD participants were male and the mean age was 33 years (±19) (see **Table 1** for demographic information). One hundred forty-three (88.8%) reported a single bleeding disorder diagnosis; 18 (11.2%) reported multiple diagnoses (see **Table 2** for diagnoses). The most commonly used treatments for hemophilia A or B were recombinant clotting factor concentrate (factor) (79.5%), topical or non-plasma products (e.g., desmopressin) (15.1%), and bypassing agents (12.3%). Non-plasma or topical products (51.9%), plasma-derived factor VIII (48.2%), and hormonal contraceptives (25.9%) were used to treat von Willebrand disease. Treatment characteristics are displayed in **Table 3**. Over 77% of those using intravenous product infused at home; 58.3% self-infused. Of those using factor, 47.9% used it to prevent bleeds, with 38.7% practicing continuous prophylaxis. In terms of health care utilization **(Table 4)**, 30.4% of participants did not visit a healthcare provider regularly for their bleeding disorder; top reasons included no need for regular care (53.2%) and expense (23.9%). One hundred (63.3%) respondents had one (35.4%) or more (64.6%) healthcare providers from whom they usually received care for their bleeding disorder. Nearly 89% visited a hematologist; when a hematologist was not visited, a family practitioner was the next most common provider used (32.3%). The most common places for care were the doctor's office (49.7%), ER (15.1%), and hospital outpatient department (13.2%). In the last 12 months, 32.6% visited an ER and 18.5% were admitted to a hospital due to their bleeding disorder; 28.8% delayed or did without needed care due mostly to expense/lack of insurance (54.8%), lack of healthcare providers (21.4%), or inconvenience (19.1%). Children were more likely than adults to visit a healthcare provider regularly for care of their bleeding disorder (100.0% vs. 73.6%, p<.0001); see a healthcare provider for care of their bleeding disorder at least once a year (97.8% vs. 53.1%, p<.0001); report actually having seen a healthcare provider for their bleeding disorder in the past year (97.8% vs. 65.2%, p<.0002); and use recombinant factor (hemophilia A or B) (96.0% vs. 70.8%, p=.01). Children were less likely than adults (7.0% vs. 42.2%, p<.0001) to have delayed or done without care in the past year. Females were less likely than males to see a healthcare provider regularly for their bleeding disorder (72.1% vs. 90.7%, p<.005) and also see them less frequently - only 48.1% of females vs. 81.7% of males see a healthcare provider for their bleeding disorder at least once a year, with 37.7% of females vs. 8.5% of males seeking care only prior to a medical procedure that may make them bleed (p<.0001). Females using infusible treatment products also were less likely to practice home infusion (60.7% vs. 85.1%, p<.01). Delaying or doing without care due to expense/lack of insurance was not statistically associated with current insurance status or with poverty status, but was associated with income (p<=0.02). At least 9.0% of participant households are in poverty. The expense of care led to delays among all income groups except the highest (data not shown).

Conclusions

Preliminary data from CHOICE offers new insights in the treatment and healthcare utilization among non-HTC PWBD. The majority of participants received treatment for their bleeding disorder and reported care from an appropriate healthcare provider; however, about 18% with needs did not see a healthcare provider regularly and over one-third delayed or did without care for their bleeding disorder. Adult participants were significantly less likely to regularly visit a healthcare provider for care of their bleeding disorder than child participants. Additional analysis of disease severity, treatment, and healthcare utilization is needed to determine unmet healthcare needs of non-HTC PWBD and how to address these needs through planning and programming. Further, analysis of health outcomes will be important to determine whether there are differences among participants based on treatment, healthcare utilization, and other factors. Additional work is needed to determine whether the health outcomes of non-HTC PWBD differ from other PWBD. This sample does not necessarily represent all non-HTC PWBD, as affiliation with an HTC was not always clear and targeted outreach by HFA Member Organizations in some regions may have led to over-representation of some participant characteristics.

References

1 Baker JR, Crudder SO, Riske B, Bias V, Forsberg A. A Model for a Regional System of Care to Promote the Health and Well-Being of People with Rare Chronic Genetic Disorders. Am J Pub Health 2005;95(11):1910-16.

2 Soucie JM, Evatt B, Jackson D. Occurrence of hemophilia in the United States. The Hemophilia Surveillance System Project Investigators. Am J Hematol 1998;59:288-94.

3 Soucie JM, Nuss R, Evatt B, Abdelhak A, Cowan L, Hill H, Kolakoski M, Wilber N. Mortality among males with hemophilia: relations with source of medical care. The Hemophilia Surveillance System Project Investigators. Blood 2000;96:437-42.

4 Soucie JM, Symons J, Evatt B, Brettler D, Huszti H, Linden J and the Hemophilia Surveillance System Project Investigators. Home-based factor infusion therapy and hospitalization for bleeding complications among males with hemophilia. Haemophilia 2001;7:198–206.

5 Baker JR, Riske B, Drake JH, Forsberg AD, Atwood R, Voutsis M, Shearer R. US Hemophilia Treatment Center population trends 1990–2010: patient diagnoses, demographics, health services utilization. Haemophilia 2013; 19: 21-6.

6 Copyright (c) 2002-2010 by SAS Institute Inc., Cary, NC, USA.

TABLE 1

Demographic characteristics for non-HTC PWBD (n=161)

	#	%
Age (years)		
<18	46	28.75
18+	114	71.25
Mean/Median (range, SD)	33/34 (0-71, 19.51)	
Sex		
Female	78	48.45
Male	83	51.55
Race		
White	121	78.06
Asian	7	4.52
American Indian/Alaska Native, Black or African American, or Native Hawaiian or Other Pacific Islander	6	3.88
Other	11	7.11
Prefer not to answer	10	6.45
Ethnicity		
Hispanic	44	28.03
Non-Hispanic	108	68.79
Prefer not to answer	5	3.18
Type of insurance		
Commercial or private insurance	82	53.25
Medicaid, Medicare, or Both	35	22.73
Commercial or private insurance plus Medicaid, Medicare, Both or Other	10	6.49
Medicaid, Medicare, or Both plus Other	5	3.25
Other	9	5.84
Uninsured	10	6.49
Prefer not to answer	3	1.95
Employment status, respondents >=18 years old		
Employed full-time	52	50.48
Employed at least part-time	8	7.76
Homemaker, student, retired, or other	22	21.36
Permanently or temporarily disabled	17	16.5
Prefer not to answer	4	3.88
Not applicable	56	
Household income		
< $25,000	33	20.75
$25,000 - $49,999	34	21.38
$50,000 - $74,999	21	13.21
$75,000 - $99,999	20	12.58
>= $100,000	30	18.87
Prefer not to answer	21	13.21
Estimate of households meeting HHS poverty guideline*	12	9.02

*Calculated using top of income category

TABLE 2

Diagnoses of non-HTC PWBD (n=161)

	#	%
Hemophilia A alone or in combination (n=80)		
Severe	48	60.00
Moderate	7	8.75
Mild	21	26.25
Do not know severity	4	4.44
Hemophilia B alone or in combination (n=22)		
Severe	6	27.27
Moderate	5	22.73
Mild	11	50.00
Von Willebrand disease alone or in combination (n=48)		
Type 1	31	64.58
Type 2	9	18.74
Other type or unknown	8	16.67
Platelet disorder alone or in combination (n=17)		
Platelet storage pool defect	6	35.29
Inherited thrombocytopenia, other or unknown hereditary functional platelet disorder	11	64.69
Other clotting factor deficiency or unspecified bleeding disorder alone or in combination (n= 18)		

TABLE 3

Treatment characteristics of non-HTC PWBD who used any medications, surgeries, or other modalities to treat their bleeding disorder in the last 12 months (n=121)

	#	%
Treatment products used by those with hemophilia A or B alone (n= 73)*		
Recombinant clotting factor concentrate	58	79.45
Plasma-derived clotting factor concentrate (FVIII) containing VWF	7	9.59
Monoclonal or porcine clotting factor concentrate or blood bank product	5	6.85
Bypassing agents	9	12.33
Non-plasma or topical agents	11	15.07
Hormonal contraceptives or gynecological surgery or procedure	5	6.85
Other surgery or procedure	5	6.85
Treatment products used by those with von Willebrand disease (VWD) alone (n= 27)*		
Plasma-derived clotting factor concentrate (FVIII) containing VWF	13	48.15
Non-plasma or topical agents	14	51.85
Hormonal contraceptives or gynecological surgery or procedure	7	25.92
Other	2	7.41
Treatment products used by those with a platelet disorder disorder alone (n= 6)*		
Non-plasma or topical agents	5	83.33
Other	3	50.00
Home infusion		
Does not take products by infusion	23	19.49
No	21	17.80
Yes	74	62.71
Who performs home infusion*		
Self-infuses at least some times	42	58.33
Self-infuses exclusively	33	45.83
A family member performs infusions at least some times	32	44.44
A family member performs infusions exclusively	23	31.94
A healthcare provider, home health aid or someone else performs infusions at least some times	9	12.50
A healthcare provider, home health aid or someone else performs infusions exclusively	6	8.33
Clotting factor used in the last 12 months		
Yes	92	76.03
No	29	23.97
Use clotting factor to prevent bleeds		
Yes	34	47.89
No	37	52.11
Practice continuous prophylaxis		
Yes	24	38.71
No	38	61.29

*more than one response is allowed

TABLE 4

Healthcare utilization among non-HTC PWBD (n=161)

	#	%
Do you see a healthcare provider regularly for care of your bleeding disorder?		
Yes	112	69.57
No	49	30.43
Reason for not seeing a healthcare provider regularly*		
I do not need to seek care regularly	25	53.19
The care is too expensive	11	23.91
The healthcare providers who are available will not treat me for my bleeding disorder	5	10.87
I do not like, or I lack confidence in, the healthcare providers who are available to me	7	15.22
There are no healthcare providers who treat bleeding disorders in my area	4	8.70
I do not know where to get care	4	8.70
Clinic/office hours are inconvenient or transportation problems	5	10.87
Other	4	8.70
Are there one or more healthcare providers you usually see for care of your bleeding disorder?		
Yes	100	63.29
No	58	36.71
Healthcare professionals usually seen for bleeding disorders care*		
Hematologist	88	88.89
Family practitioner/General practitioner/Internist	32	32.32
Pediatrician	19	19.19
Obstetrician/Gynecologist	13	13.13
Oncologist	10	10.10
Orthopedist/Orthopedic surgeon	9	9.09
Nurse/Nurse practitioner/Physician assistant	10	10.10
Physical therapist	8	8.08
Ear, nose and throat doctor (ENT); Otolaryngologist, Otorhinolaryngologist	6	6.06
Social worker	6	6.06
About how often do you see a healthcare provider for your bleeding disorder?		
At least once a year	104	65.41
At least once every 2-3 years	16	10.06
At least once every 4 or more years	3	1.89
Only prior to a medical procedure that will cause me to bleed	36	22.64
When did you last see a healthcare provider about your bleeding disorder?		
Within the past year	118	74.21
1-2 years ago	22	13.84
3-4 years ago	3	1.89
More than 4 years ago	16	10.06
Where do you usually go for care of your bleeding disorder?		
Doctor's office	79	49.69
Hospital emergency room	24	15.09
Hospital outpatient department	21	13.21
Hemophilia Treatment Center (HTC)	14	8.81
Do not go to one place most often	7	4.40
Home	5	3.14
Clinic or Community Health Center	5	3.14
Do not know	4	2.52
In the last 12 months, have you delayed or done without care or treatment for your bleeding disorder?		
Not applicable - did not need care in the last 12 months	15	9.38
Yes	46	28.75
No	99	61.88
Reason for delaying or doing without care*		
Care or treatment is too expensive or no insurance	23	54.76
There are no healthcare providers who treat bleeding disorders in my area	9	21.43
It was inconvenient to treat right away	8	19.05
I have concerns about using the products available to treat my bleeding disorder	6	14.29
I cannot find a healthcare provider that accepts my insurance	5	11.90
Transportation problems	3	7.14
I do not know how to infuse	4	9.52
Healthcare provider or insurance delay or denial of care	4	9.52
Other	2	4.76
In the last 12 months, have you gone to a hospital emergency room because of your bleeding disorder?		
Yes	42	32.56
No	87	67.44
Number of emergency room visits due to bleeding disorder in last 12 months: Mean/Median (range, SD)	3.1/2 (1-20, 3.45)	
In the last 12 months, have you been admitted to a hospital because of your bleeding disorder?		
Yes	27	18.49
No	119	81.51
Number of hospital admissions due to bleeding disorder in the last 12 months: Mean/Median (range, SD)	1.52/1 (1-3, 0.79)	
Number of nights spent as an inpatient due to bleeding disorder in the last 12 months: Mean/Median (range, SD)	6.91/3 (1-45, 10.15)	

*more than one response is allowed

Publication Number 4761 – Submission ID 97474

This work was supported by Cooperative Agreement number 1U27DD000859 from the Centers for Disease Control and Prevention (CDC). The findings and conclusions in this report are those of the authors and do not necessarily represent the official position of the CDC.