Public Health Surveillance of People Not Receiving Care at US Federally-funded Hemophilia Treatment Centers: Methods and Demographics of the CHOICE Project

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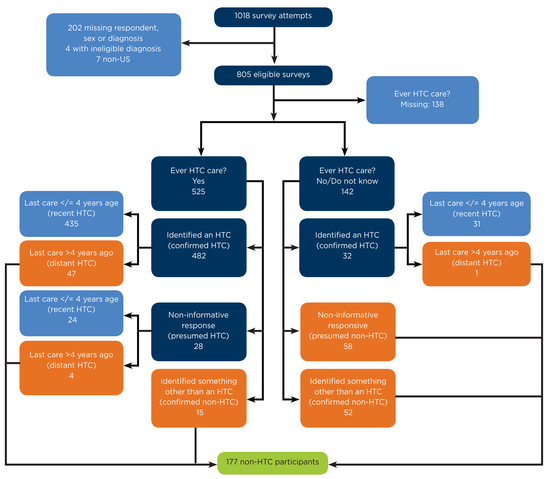
Introduction and Objectives:

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 this population[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 persons with hemophilia (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 unestimated number of persons with other genetic bleeding disorders, receive care elsewhere. Data on these persons with bleeding disorders (non-HTC PWBD) are not collected on a regular basis nor maintained in a central database. Consequently, little is known about their characteristics, care, health status, and needs. 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.

Figure 1. Algorithm for determining Non-HTC status of CHOICE participants



Methods:

HFA and CDC collaborated on the development of the CHOICE survey questions with the input of members of the bleeding disorders community. Survey elements included diagnosis, treatment regimen and treatment products used, inhibitor status, joint function and disease (invasive orthopedic procedures, use of pain medication), bleeding history, HIV and hepatitis infection and other comorbidities, health services utilization (usual source of care, frequency of care, barriers to regular care, delay of care, emergency room utilization and hospitalizations within the past 12 months), demographics, and patient satisfaction (RAND PSQ-18).

HFA learned through its promotion of CHOICE to the bleeding disorders community that many people in the community do not know whether they receive their care and services from an HTC. Thus, a series of questions was included in the CHOICE survey to help establish objectively whether a respondent received care at an HTC. Respondents were asked whether they had ever attended an HTC. Regardless of whether they responded "yes" or "no" to this question, they were referred to a list of institutions consisting of current and past federally-funded HTCs and asked to identify those they had attended. If they had not received treatment at any of the institutions on the list, they were asked to provide the name, city, and state of the provider from whom they receive care. For purposes of comparison with HSS and other surveillance data, participants also were asked when they had last received care at an HTC (or elsewhere). Participants' status as non-HTC PWBD was assigned using an algorithm based on their response to these questions **(Figure 1)**.

From 04/2013-07/2015, US PWBD 18 years old or older (adults) and caregivers of children with bleeding disorders were recruited to take the approximately 20-minute CHOICE survey in English or Spanish, online or on paper. Non-HTC PWBD were specifically solicited but others were not discouraged from participation. Recruitment of non-HTC PWBD participants was supported by those who may interact with them, including healthcare providers, homecare companies, and local and regional organizations serving PWBD. In addition, HFA recruited non-HTC PWBD to take the CHOICE survey via direct face-to-face communication and social media. The survey was offered at HFA's annual Symposium and at events sponsored by HFA Member Organizations in online and paper formats. Participants could also request that a paper version of the survey be mailed to them by HFA.

Results and Conclusions:

One thousand eighteen survey attempts were made; 213 were judged ineligible for inclusion. Of the 805 surveys retained, 752 (93%) were completed in English and 53 (7%) were completed in Spanish; 783 (97%) were completed electronically and 22 (3%) on paper. Twenty-two percent of participants (177) were non-HTC PWBD **(Figure 1)**. See **Table 1** for diagnoses, **Table 2** for demographic information. Non-HTC PWBD hailed from 39 states **(Figure 2)**; six states accounted for 55% of participants: Texas (31, 18%), California (25, 14%), Florida (12, 7%), New York (11, 6%), Massachusetts (10, 6%) and Illinois (9, 5%). Compared to the US general population, the non-HTC participants responding to this survey represent a more ethnically diverse population (27% vs. 17% Hispanic) and are proportionately less African American (<4% vs. 13%)[6].

Preliminary data from the CHOICE Project demonstrate the strengths and challenges of using direct patient survey to identify PWBD receiving care outside the federally-funded network of HTCs. However, this sample does not necessarily represent all non-HTC participants, 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; further analysis is needed to determine whether this was the result of recruitment methods or the underlying population structure. The CHOICE patient outreach/recruitment methodology may be helpful in identifying pockets of PWBD whose health experiences may be underrepresented in clinic-based surveillance and other data collection efforts. Additional work is needed to analyze the health outcomes of non-HTC PWBD and to determine whether those outcomes are different from other PWBD as well as to understand how best to identify and recruit non-HTC PWBD for surveillance.

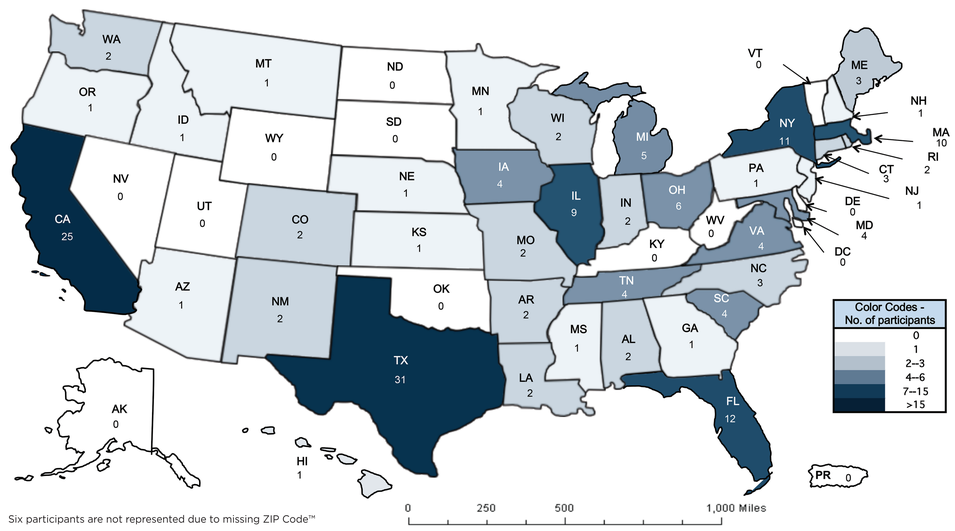
Table 1. Diagnoses and clinical characteristics of non-HTC PWBD (n=177)

	#	%
Hemophilia A alone	**79**	**44.63**
Severe	53	67.09
Moderate	9	11.39
Mild	16	20.25
Do not know severity	1	1.27
Hemophilia B alone	**23**	**12.99**
Severe	6	26.09
Moderate	5	21.74
Mild	12	52.17
Von Willebrand disease alone	**37**	**20.90**
Type 1	24	64.86
Type 2	7	18.92
Other type or unknown	6	16.22
Platelet disorder alone	**11**	**6.21**
Other bleeding disorder alone	**6**	**3.39**
More than one bleeding disorder diagnosis	**21**	**11.86**
Age at diagnosis		
Before birth or within 24 hours of birth	26	14.94
More than 24 hours after birth	148	85.06
Age at diagnosis for those diagnosed >24 hours after birth (n=148)		
Range	2 days-60 years	
Mean	13.74 years	
Median	6.5 years	
1st quartile	7 months	
2nd quartile	25 years	

Table 2. Demographic characteristics and usual place of care for non-HTC PWBD (n=177)

	#	%
Age (years)		
0-10	25	14.20
11-19	29	16.48
20-44	69	39.20
45-64	44	25.00
65+	9	5.11
Mean/Median (range)	33/32 (0-71)	
Sex		
Female	84	47.46
Male	93	52.54
Race		
White	130	76.92
Asian	10	5.92
American Indian/Alaska Native, Black or African American, or Native Hawaiian or Other Pacific Islander	7	4.14
Other	11	6.51
Prefer not to answer	11	6.51
Ethnicity		
Hispanic	46	26.74
Non-Hispanic	118	68.60
Prefer not to answer	8	4.65
Type of insurance		
Commercial or private insurance	87	51.79
Medicaid, Medicare or Both	36	21.43
Commercial or private insurance plus Medicaid, Medicare, Both or Other	13	7.74
Medicaid, Medicare or Both plus Other	6	3.57
Other	9	5.36
Uninsured	12	7.14
Prefer not to answer	5	2.98
Employment status, respondents >=18 years old		
Employed full-time	56	48.70
Employed at least part-time	11	9.57
Homemaker, student, retired or other	25	21.73
Permanently or temporarily disabled	18	15.65
Prefer not to answer	5	4.35
Not applicable (respondent for child <18 years old)	60	
Usual place of care		
Doctor's office	86	49.43
Hospital emergency room	28	16.09
Hospital outpatient department	21	12.07
Hemophilia Treatment Center (HTC)	14	8.05
Do not go to one place most often	8	4.60
Home	7	4.02
Clinic or Community Health Center	5	2.87
Do not know	5	2.87

Figure 2. Distribution of CHOICE participants assigned non-HTC status, by state



Six participants are not represented due to missing ZIP Code™

References and required disclaimer:
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[6] U.S. Census Bureau; American Community Survey, 2014 American Community Survey 1-Year Estimates, Tables B02001, C03002; generated by Meredith Oakley; using American FactFinder; <http://factfinder2.census.gov>; (11 July 2016).
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