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Community counts: Evolution of a national surveillance system for bleeding disorders

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To the Editor:

Comprehensive public health surveillance of hemophilia and related disorders is important to monitor health indicators to inform prevention strategies. The United States (US) Centers

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AUTHOR CONTRIBUTIONS

Marilyn Manco-Johnson designed research, performed research, analyzed data, and wrote the paper; Vanessa Byams designed research, performed research, and wrote the paper; Michael Recht designed research, performed research, edited and approved the manuscript; Becky Dudley coordinated the research project, performed research, reviewed and approved the manuscript; Brandi Dupervil analyzed data, reviewed and approved the paper; Diane Aschman performed research, reviewed and approved the paper; Meredith Oakley designed research, performed research, reviewed and approved the manuscript; Suzanne Kapica performed research, reviewed and approved the manuscript; Mariam Voutsis performed research, reviewed and approved the manuscript; Steven Humes performed research, reviewed and approved the manuscript; Roshni Kulkarni performed research, reviewed and approved the manuscript; Althea Grant designed research, performed research, reviewed and approved the manuscript.

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CONFLICT OF INTEREST

Marilyn J. Manco-Johnson has no conflicts to declare. Vanessa Byams has no conflicts to declare. Michael Recht has no conflicts to declare. Becky Dudley has no conflicts to declare. Brandi Dupervil has no conflicts to declare. Diane Aschman has no conflicts to declare. Meredith Oakley has no conflicts to declare. Suzanne Kapica has no conflicts to declare. Mariam Voutsis has no conflicts to declare. Steven Humes has no conflicts to declare. Roshni Kulkarni has no conflicts to declare. Althea Grant has no conflicts to declare.

for Disease Control and Prevention (CDC) Haemophilia Surveillance Study (HSS,1993–1998) was population based in six states to estimate occurrence rates, sources of care, complications, and outcomes.[1] HSS reported 67% of the 16 960 persons with hemophilia A or B projected to be then living in the US were seen at hemophilia treatment centers (HTCs).[1] HSS determined a case rate for intracranial hemorrhage of 0.0054 cases/patient year, with increased risk with the human immunodeficiency virus (HIV) coinfection.[2] Persons with hemophilia and HIV or acquired immunodeficiency syndrome (AIDS) had an increased risk of death (5- and 33-fold risk as compared to uninfected persons, respectively); mortality was decreased by 40% in persons seen in HTCs.[3]

Informed by HSS, the Universal Data Collection (UDC) project (1998–2011) collected data on 27 368 individuals (19 023 with hemophilia A or B) seen within the 129 HTCs of the US HTC network (USHTCN). UDC focused on treatment-related infections and arthropathy.[4] No new infections of HIV or hepatitis were linked to products used to treat bleeding disorders in 90 000 tested specimens.[4] UDC documented the rapid penetration of prophylaxis and associated decreases in bleeding and arthropathy.[5]

In 2012, hemophilia surveillance was revamped as the Community Counts (CC) project to focus on contemporary outcomes and complications. With novel therapies, widespread prophylaxis, and highly effective HIV and hepatitis C treatments, persons with hemophilia now survive to be at risk for disorders of aging, and the development of inhibitory antibodies has emerged as a major cause of morbidity. CC builds upon previous work while addressing current priorities relevant to persons affected by bleeding disorders.

CC represents an innovative and effective partnership among: (1) a healthcare network (the USHTCN); (2) a national data coordinating center [the American Thrombosis and Hemostasis Network (ATHN)]; (3) a national public health agency (CDC); and (4) community participants, to apply public health surveillance techniques to a set of rare chronic diseases.

The USHTCN includes 139 HTCs located primarily in academic medical centers and partially funded by the Health Resources and Services Administration. The USHTCN is organized in 10 geographic regions covering the continental US, Alaska, Hawaii, Guam, and Puerto Rico with leadership through regional coordinators (with nursing, social work, or public health expertise) who function as information and support conduits and regional directors (mainly hematologists) who assume responsibility for the scientific direction. ATHN was established by USHTCN members to create and manage a secure data resource. ATHN, as the coordinating center, provides the electronic data platform, training and technical assistance. CDC provides funding, programmatic guidance, laboratory testing, technical assistance, and data management, analysis, and reports. The HTCs identify and enroll eligible participants, collect specimens, and analyze, present, and publish results. CC infrastructure includes five core committees. Each committee includes representatives of ATHN, CDC, and the USHTCN. The Executive Committee provides overall direction of the project. The Administrative Committee supports the administration of CC throughout the regions. The Science Committee develops and evaluates the science of the project. A Project Review Committee evaluates proposals for analysis based on feasibility and scientific

validity. A Presentation and Publication Committee reviews all project products for scientific soundness and authorship.

The HTC PP captures minimal data to estimate population size and characteristics of patients receiving care within the USHTCN. HTC PP is a Health Insurance Portability and Accountability Act of 1996 (HIPAA) compliant de-identified dataset and includes: year of birth, gender, race, ethnicity, 3-digit zip code, insurance status, primary diagnosis, laboratory assays for factor deficiency, and history of HIV and/or HCV infection. Persons with inherited bleeding disorders, Ehlers-Danlos syndrome and venous thromboembolism (VTE) are included.

The Registry includes a HIPAA compliant limited dataset and a sample repository. VTE and Ehlers-Danlos syndrome are excluded. The outcomes and complications collected include: bleeding events; joint surgeries; inhibitor development; treatment products and regimens; blood-borne infections; diseases of aging (hypertension, cardiovascular disease, cancer, etc); functional impairment; pain and health service utilization. A central laboratory located at the CDC utilizes advanced technology to detect very low levels of inhibitory antibodies, even in the presence of exogenous factor, as well as non-neutralizing binding antibodies that may precede or contribute to inadequate treatment response.[6] Risk-based testing is performed for blood-borne pathogens.

Mortality Reporting collects causes of death of persons with bleeding disorders as a HIPAA compliant de-identified dataset.

ATHN processes incorporate administrative, technical, and physical safeguards for the web-based data collection system to prevent inappropriate use or disclosure of data to ensure HIPAA/Health Information Technology for Economic and Clinical Health Act of 2009 compliance. Data are securely transmitted to the CDC daily.

From September 30,2011 through September 30,2017,168 644 visits were recorded on 70 137 individuals in HTC PP; the Registry recorded data on 13 198 persons, including 41% of patients in HTC PP with hemophilia A and B (Table 1). Mortality data were collected on 822 decedents.

Public health surveillance of bleeding disorders is essential because complications are of high prevalence, economic impact, and morbidity, and are often preventable. A strength of CC is its representativeness, covering the entire US and territories; race and ethnicity approximate the general US population. By accruing baseline and longitudinal data spanning the entire health history of individuals, CC will yield invaluable information on key outcomes.

Limitations to CC surveillance include cost, training, quality assurance, and reliance on self-assessment for patient reported outcomes. In spite of these limitations, CC is a model public health surveillance program for rare chronic diseases. With the proliferation of new treatment products and regimens, CC is likely to facilitate an exponential growth in data about hemophilia practices and outcomes to inform treatment recommendations.

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TABLE 1

HTC Population Profile (n = 70 137) and Registry (n = 13 198) participant characteristics

	HTC PP	Registry
Gender		
Male	39 494 (56)	10 538 (80)
Female	30 643 (44)	2660 (20)
Race		
White	57 015 (81)	10 653 (81)
Black	8153 (12)	1483 (11)
Asian	1916 (3)	431 (3)
Unknown	1734 (3)	299 (2)
Mixed race	591 (1)	174 (1)
American Indian or Alaska Native	482 (1)	102 (1)
Native Hawaiian or Other Pacific Islander	246 (0)	56 (0)
Ethnicity		
Not Hispanic, Latino/a, or Spanish origin	59 353 (85)	11 205 (85)
Hispanic, Latino/a, or Spanish origin	9736 (14)	1847 (14)
Unknown	1048 (2)	146 (1)
Age	27 (mean); 20 (median); Range 0–90+	25 (mean); 19 median; Range 0–104
<2 years	2552 (4)	421 (3)
2–10 years	12 100 (17)	2953 (22)
11–19 years	18 869 (27)	3467 (26)
20–44 years	20 145 (29)	4035 (31)
45–64 years	11 193 (16)	1747 (13)
65+ years	5278 (8)	575 (4)
Health insurance		
Insured	67 696 (97)	12 762 (97)
Uninsured	1892 (3)	388 (3)
Unknown	549 (1)	48 (0)
Diagnosis		
Bleeding Disorder, no laboratory diagnosis	1170 (2)	22 (0)
Hemophilia A (FVIII)	17 115 (24)	7565 (57)
Hemophilia B (FIX)	5396 (8)	1986 (15)
Other (rare clotting factor deficiencies and inherited or functional platelet disorders)	9524 (14)	826 (6)
VTE	19 140 (27)	N/A
VWD	17 792 (25)	2799 (21)