Joint Outcomes in United States (U.S.) Hemophilia Patients: A Report of the Community Counts Registry

Marilyn J. Manco-Johnson[1], Michael Recht[2], Roshni Kulkarni[3], Brandi Dupervil[4], Vanessa R. Byams[4], Becky Dudley[5], Diane J. Aschman[5], Mariam Voutsis[6], Steven Humes[7] and Meredith Oakley[4]

[1]Hemophilia & Thrombosis Center, University of Colorado Anschutz Medical Campus and Children's Hospital Colorado, Aurora, USA; [2]The Hemophilia Center, Oregon Health & Science University, Portland, OR, USA; [3]Center for Bleeding and Clotting Disorders, Michigan State University, East Lansing, MI, USA; [4]Division of Blood Disorders, National Center on Birth Defects and Developmental Disabilities, Centers for Disease Control and Prevention, Atlanta, GA, USA ; [5]American Thrombosis and Hemostasis Network, Riverwoods, IL, USA; [6]Regional Comprehensive Treatment Center, Icahn School of Medicine at Mount Sinai, New York, NY, USA; [7]Hemophilia Diagnostic and Treatment Center, University of North Carolina at Chapel Hill, Chapel Hill, NC, USA

Introduction and Objectives

Arthropathy causes substantial pain, disability and decreased quality of life in persons with hemophilia; the goal of prophylaxis is to prevent/decrease bleeding and morbidity related to arthropathy. Patient outcomes relative to hemophilia severity and past joint disease in those enrolled in the Community Counts Centers for Disease Control and Prevention (CDC) Public Health Surveillance for Bleeding Disorders Project (Community Counts) were analyzed.

Materials and Methods

Using the American Thrombosis and Hemostasis Network (ATHN) as a coordinating center and utilizing standardized collection tools, authorized data on eligible patients with hemophilia enrolled in the Community Counts Registry for Bleeding Disorders Surveillance (the Registry) at the U.S. Hemophilia Treatment Centers Network (USHTCN) were sent to the CDC. Baseline data on demographics, treatment and outcomes related to arthropathy were extracted for this analysis. The Registry is a subset of Community Counts participants and collects longitudinal, patient-level data on USHTCN patients with bleeding disorders along with blood samples based on their risk for blood-borne infections and inhibitor development.

Results

Data from 5,025 males with hemophilia A and B were analyzed. Of these, 4,016 (80%) have hemophilia A; and 2,837 (56%) have severe hemophilia A (2,419/85%) or B (418/15%). Only 4% of the 2,837 with severe hemophilia A or B are less than 2 years of age; 26% are from 2–10, 25% from 11–19, 34% from 20–44 and 11% over 44. The racial distribution of this group is 76% white, 15% black, and 4% Asian. 86% are not of Hispanic, Latino/a or Spanish origin.

Almost 2/3 of Registry patients 20 years old or older use continuous prophylaxis. In spite of this, substantial morbidity continues to be documented in this group, as evidenced by participation in school and work, chronic pain, opioid use and requirement for invasive joint procedures. However, the outcomes of persons using early prophylaxis and those with tolerized inhibitors were quite good in comparison.

Discussion/Conclusions

The U.S. Community Counts Registry allows determination of trends in patient outcomes as related to disease severity and treatments. This analysis discloses that despite awareness and high utilization of prophylaxis, significant morbidity persists, including joints with multiple/recurrent hemorrhages, significant pain and joint surgeries. These data underscore the importance of prevention of joint bleeding in hemophilia to reduce joint morbidities. The Community Counts project allows tracking of outcomes and prevention efforts to increase primary prophylaxis and early immune tolerance for severe hemophilia.

Table 1. Outcomes of males 20 years old and older with severe hemophilia by treatment regimen (n=1,280)

Treatment Regimen	Primary Prophylaxis (n=34)	Prophylaxis-Continuous (n=777)	Episodic (On-Demand) (n=390)	Prophylaxis-Continuous with By-Passing Agents Plus ITI (n=10)	Prophylaxis-Event-Based, Short-Term or Intermittent (n=94)	Immune Tolerance Induction (n=9)
% used treatment type	2.66%	60.7%	30.5%	0.78%	7.34%	0.70%
# of severe males that have ever had >/= 4 joint bleeds in one joint	16	634 (49.5%)	324 (25.3%)	9	79	8
Mobility						
Unrestricted school or work and unrestricted recreational activities	65%	35%	27%	20%	26%	44%
Unrestricted school or work with limited recreational activity levels	18%	25%	21%	30%	18%	11%
Limited school or work and limited recreational activity levels	12%	29%	34%	50%	32%	11%
Limited school or work, limited recreational activity levels and limited self-care activity levels	6%	7%	12%	0%	20%	22%
Requires assistance from another person for school or work or self-care and unable to participate in recreation	0%	2%	3%	0%	2%	11%
Opioid usage in last 12 months						
Yes	55%	49%	56%	29%	61%	71%
Frequency of opioid usage						
Every day	50%	41%	49%	50%	48%	40%
Most days	17%	16%	15%	0%	17%	20%
History of joint procedure						
Yes	21%	48%	49%	50%	52%	56%

* Prophylaxis (Prophy): Use of any treatment product on a regular basis to prevent any bleeds or to maintain tolerance to factor or both (even if adherence to the regimen is not perfect), that is expected to continue indefinitely. (For Community Counts, primary prophylaxis is defined as regular continuous treatment initiated in the absence of documented joint disease started before the second clinically evident large joint bleed and age 3 years.)

Table 2. Outcomes of severe hemophilia A and B patients with a history of inhibitor[Ŧ] by tolerized status and treatment regimen (n=530)

	Likely Tolerized* (n=382)						Active Inhibitor[§] (n=148)					
	Primary Prophylaxis (n=83)	Prophylaxis-Continuous (n=314)	Prophylaxis-Continuous with By-Passing Agents Plus ITI (n=3)	Prophylaxis-Event-Based, Short-Term or Intermittent (n=12)	Immune Tolerance Induction (n=16)	Episodic (On-Demand) (n=37)	Primary Prophylaxis (n=46)	Prophylaxis-Continuous (n=100)	Prophylaxis-Continuous with By-Passing Agents Plus ITI (n=7)	Prophylaxis-Event-Based, Short-Term or Intermittent (n=4)	Immune Tolerance Induction (n=19)	Episodic (On-Demand) (n=18)
% used treatment type		**82.2%**	**0.79%**	**3.14%**	**4.19%**	**9.7%**		**67.6%**	**4.73%**	**2.70%**	**12.84%**	**12.2%**
Mobility												
Unrestricted school or work and unrestricted recreational activities	83%	68%	67%	42%	88%	43%	76%	65%	57%	25%	84%	22%
Unrestricted school or work with limited recreational activity levels	10%	16%	33%	25%	6%	11%	15%	15%	29%	50%	11%	6%
Limited school or work and limited recreational activity levels	4%	11%	0%	8%	6%	38%	4%	12%	14%	0%	0%	33%
Limited school or work, limited recreational activity levels and limited self-care activity levels	1%	2%	0%	17%	0%	5%	4%	7%	0%	25%	0%	22%
Requires assistance from another person for school or work or self-care and unable to participate in recreation	0%	0%	0%	0%	0%	3%	0%	1%	0%	0%	0%	11%
Chronic pain in the last 12 months												
Yes	10%	22%	0%	50%	19%	62%	11%	23%	14%	100%	26%	67%
Frequency of chronic pain in the last 12 months												
Every day	13%	28%	0%	33%	33%	26%	20%	35%	100%	25%	20%	67%
Most days	13%	28%	0%	17%	33%	39%	0%	13%	0%	0%	0%	8%
Opioid usage in last 12 months												
Yes	40%	39%	0%	67%	67%	52%	38%	52%	100%	75%	20%	67%
Every day	0%	44%	0%	25%	100%	50%	33%	17%	100%	67%	0%	50%
Most days	0%	15%	0%	25%	0%	17%	33%	33%	0%	0%	0%	0%
History of joint bleed												
Yes	67%	87%	67%	67%	56%	100%	78%	90%	71%	100%	68%	94%
>/= 4 joint bleeds in one joint	25%	57%	50%	63%	56%	92%	36%	53%	40%	100%	54%	82%
History of joint procedure												
Yes	3%	19%	0%	42%	6%	46%	2%	19%	14%	50%	16%	39%

Ŧ These are hemophilia patients with a history of inhibitor currently using FVIII or FIX concentrates or bypassing agents for bleeding events that require treatment.
* Likely Tolerized is defined as having a history of inhibitor and currently using FVIII or FIX concentrates for bleeding events that require treatment.
§ Active Inhibitor is defined as having a history of inhibitor and currently using bypassing agents for bleeding events that require treatment.

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72 Treasure Lane, Riverwoods, IL 60015
Phone: 800-360-2846
www.athn.org

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