Poster #15

Real-world treatment patterns, health outcomes, and healthcare resource use among persons with hemophilia A

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Summary

Persons with hemophilia A (PwHA) are medically complex.

Treatment patterns, clinical outcomes, and healthcare resource use among PwHA vary by age group.

This study assessed the association of age with outcomes and healthcare resource use in PwHA, which may help inform management approaches.

Older PwHA had higher comorbidity rates, used on-demand treatment more frequently, and had more bleeding events than younger patients.

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Introduction

- Congenital hemophilia A (HA) is an inherited bleeding disorder that mostly affects males.[1]
- HA causes insufficient production of clotting factor VIII (FVIII) in the blood, resulting in poor clot formation.[2]
- About 33,000 individuals in the United States are thought to be living with hemophilia.[3]
- Life expectancy PwHA is now nearly equivalent to those without HA in high-income countries, due to improved management and treatment.[4–6]
- As with the general population, PwHA also develop comorbidities, such as joint disease, heart disease, and cancer, with these conditions developing at earlier ages than the general population, which can complicate HA management.[7,8]
- There is currently a lack of published literature describing the aging HA population.
- Understanding the characteristics of the aging HA population is required to help inform appropriate management strategies that are unique to these individuals.
- This study examined real-world patient characteristics, treatment patterns, and outcomes among treated PwHA, according to their age.

Methods

This retrospective study used real-world data from a health plan database (Figure 1).

Figure 1. Study design.

Data source
- Humana Research Claims Database; data from 01/2007 to 07/2018

Population
- Treated PwHA enrolled in commercial or Medicare Advantage Prescription Drug plans (identified using National Drug Code [NDC], Healthcare Common Procedure Coding System [HCPCS], and International Classification of Diseases codes [ICD-9, ICD-10])
  - Treatments included prophylaxis and on-demand FVIII therapy based on medication use patterns, desmopressin, or bypassing agents
- Patients were required to have 12-month continuous health plan enrollment after the index date*

Comparisons by age group (<18, 18–55, >55 years of age)
- Patient characteristics (e.g. age, region, comorbidities)
- Treatment patterns (e.g. prophylaxis, on-demand, desmopressin)
- Clinical outcomes (e.g. bleeding, arthropathy, pain)
- Healthcare resource use (e.g. inpatient hospitalization, emergency department use)

*The index date was the date of the first claim for treatment during the enrollment period.
FVIII, factor VIII; PwHA, persons with hemophilia A.

Results

Overall, 294 PwHA were included; 67 (23%) were <18 years old, 176 (60%) were 18–55 years, and 51 (17%) were >55 years (Table 1).
- The majority (59%) of patients were seen in a hemophilia treatment center (HTC).
- Of the 57 (19%) patients in whom the disease severity of HA was known, 51% had mild HA; the number of individuals classified as having severe or moderate HA was low.
- Overall, 91% of patients had ≥1 FVIII claim; the mean number of FVIII claims was highest among patients aged <18 years and lowest among those aged >55 years.

Table 1. Baseline patient demographics and characteristics.

Characteristic	Age category <18 years (*n* = 67)	18–55 years (*n* = 176)	>55 years (*n* = 51)	Overall (*N* = 294)
Mean age, years (SD)	9.7 (4.8)	33.1 (10.9)	68.3 (8.8)	33.8 (20.7)
Male, *n* (%)	66 (98.5)	167 (94.9)	48 (94.1)	218 (95.6)
US region, *n* (%)				
Northeast	<10	<10	<10	<10
Midwest	22 (32.8)	42 (23.9)	10 (19.6)	74 (25.2)
South	39 (58.2)	121 (68.8)	35 (68.6)	195 (66.3)
West	<10	10 (5.7)	<10	21 (7.1)
HTC utilization, *n* (%)	33 (49.3)	116 (65.9)	24 (47.1)	173 (58.8)

HTC, hemophilia treatment center; SD, standard deviation.

Overall, the most common comorbidities were arthritis and hepatitis C, with prevalence increasing with age (Table 2).

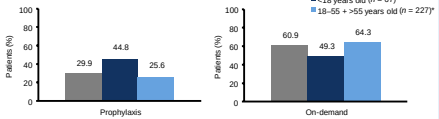
Table 2. Patient comorbidities (occurring in >5% of overall population) by age group.

Comorbidity, *n* (%)	Age category <18 years (*n* = 67)	18–55 years (*n* = 176)	>55 years (*n* = 51)	Overall (*N* = 294)
Arthritis	18 (26.9)	69 (39.2)	29 (56.9)	116 (39.5)
Hepatitis C	0	43 (24.4)	14 (27.5)	57 (19.4)
Hypertension	0	20 (11.4)	34 (66.7)	54 (18.4)
Psychiatric	<10	24 (13.6)	<10	34 (11.6)
Liver failure	0	29 (12.8)*		29 (9.9)
HIV/AIDS	<10	21 (11.9)	<10	27 (9.2)
Depression	<10	17 (9.7)	<10	26 (8.8)
COPD	0	20 (8.8)*		20 (6.8)
Diabetes	0	<10	<10	18 (6.1)
Stroke	<10	<10	<10	18 (6.1)
Ambulance	<10	12 (6.8)	<10	18 (6.1)

Numbers of <10 were suppressed to protect study subjects from potential re-identification.
*Groups were combined due to *n* < 10 in at least one group.
AIDS, acquired immune deficiency syndrome; COPD, chronic obstructive pulmonary disease; HIV, human immunodeficiency virus.

Over the 12-month period, 30% of patients had evidence of prophylactic FVIII treatment (Figure 2).
- Prophylactic FVIII treatment was more common in the group of PwHA aged <18 years (45%) compared with the combined group aged 18–55 and >55 years (26%).
- On-demand FVIII treatment was more common in the combined group of PwHA aged 18–55 and >55 years (64%) compared with the group aged <18 years (49%).
- A total of 34 patients (11.6%) had desmopressin use (more common in patients >55 years), and <10 patients had evidence of FVIII inhibitors.

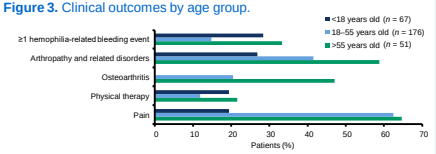
Figure 2. FVIII treatment strategy by age group.



Prophylaxis and on-demand treatment refers to FVIII treatment only, not including patients with evidence of FVIII inhibitors.
*Groups were combined due to *n* < 10 in at least one group. FVIII, factor VIII.

In the 12-month post-index period, 21% of patients overall experienced ≥1 bleeding event, 41% had evidence of arthropathy, and 53% had pain (Figure 3).
- Bleeding events were most common in patients aged >55 years (33%) and <18 years (28%) and less common in patients aged 18–55 years (15%).
- The prevalence of arthropathy and related disorders, osteoarthritis, and pain generally increased with age.

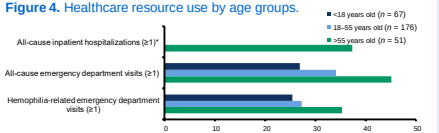
Figure 3. Clinical outcomes by age group.



Note: data for fractures and joint procedures and surgery are not shown due to small sample size (*n* < 10).

Overall, 15% of patients had ≥1 all-cause inpatient admission and 34% had an emergency department visit over the 12-month period.
- Healthcare resource use measures generally increased with age and were highest in patients aged >55 years (**Figure 4**).

Figure 4. Healthcare resource use by age groups.



Note that data for hemophilia-related inpatient hospitalizations are not shown as *n* < 10 for each age group.
*Data for <18 and 18–55 years are combined (*n* = 25; 10.3%) due to *n* < 10 in at least one group and are therefore not shown.

Conclusions

PwHA represent a medically complex group of patients.

Treatment patterns, clinical outcomes, and healthcare resource use varied by age group. Prophylactic FVIII treatment was highest among pediatric patients and on-demand FVIII treatment was highest among adults.

Older patients (aged >55 years) had the highest comorbidity burden, on-demand treatment use, and bleeding events compared with younger age groups.

This sample reflects an older age distribution than those enrolled in the Centers for Disease Control and Prevention registry or included in the HTC Population Profile.[9]

Insights on the association of age with outcomes and healthcare resource use for PwHA may help to identify opportunities for the improved management of older PwHA.

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Disclosures

EOC: Employee of Humana Healthcare Research, Inc.; **AMP:** Employee of and shareholder of stock with F. Hoffmann-La Roche Ltd./Genentech, Inc.; **RD:** Employee of Humana Healthcare Research, Inc.; **MS:** Employee of and stockholder ownership interest with Humana Inc.; **DC:** Employee of Humana Inc.; **TFS:** Employee of Humana Healthcare Research, Inc.; **RK:** Employee of Genentech, Inc.; **BTS:** Employee of Humana Healthcare Research, Inc. and stockholder ownership interest with Humana Inc.