

Web-Based Study of Patients and Caregivers in the United States and Canada: Perspectives on Improvements in Stability of Factor VIII Products for Hemophilia A

Dana DiBenedetti,¹ Theresa Coles,¹ Caroline Ling,² Tarang Sharma,³ Louisa Pericleous³

¹ RTI Health Solutions, Research Triangle Park, NC, United States; ² RTI Health Solutions, Manchester, United Kingdom; ³ Novo Nordisk A/S, Søborg, Denmark

BACKGROUND

- Hemophilia A is a rare, inherited bleeding disorder in which the affected individual lacks or has limited production of coagulation factor VIII (FVIII), resulting in the inability of the blood to clot normally. It is a recessive, sex-linked (X chromosome) disorder; thus, hemophilia A occurs almost exclusively in males.¹
- Treatment typically involves life-long replacement of FVIII through intravenous infusions to stop or prevent spontaneous or traumatic bleeds. Treatment can be provided on demand (as the bleed occurs) or given regularly as prophylaxis.² Regardless of the treatment purpose (on demand or as prophylaxis), patients must keep FVIII accessible at all times in case of a bleed. Thus, the challenge of FVIII storage and temperature stability remains constant.

OBJECTIVE

- To assess and better understand hemophilia A patients' and caregivers' experiences and preferences around FVIII storage and temperature stability attributes in the United States (US) and Canada.

METHODS

Study Design

- A cross-sectional study of English-speaking adult patients with hemophilia A and caregivers (parents) of minors/children with hemophilia A.
- Patients and caregivers were recruited from two state hemophilia organizations in the US and one national hemophilia organization in Canada
 - Postings were placed on the hemophilia organizations' Web sites, and/or e-mail invitations were sent to these organizations' member lists.
 - Two organizations posted an advertisement about the study on their Facebook page.
- Survey respondents completed several screening items to confirm their eligibility and patient or caregiver status.
- Eligible respondents then provided informed consent and were invited to complete a Web-based survey assessing patients' FVIII ordering, usage, and storage routines.

Survey Questionnaire Content

- In addition to some demographics (e.g., sex, year of birth, education), questionnaire items assessed:
 - Clinical characteristics and current product usage
 - FVIII ordering behaviors
 - FVIII storage routines and challenges
 - FVIII storage preferences
- Programming logic was included in the questionnaire to route respondents to the appropriate questions.
- Questions were tailored to respondent type (i.e., patient or caregiver).

Analytic Methods

- Data analysis was primarily descriptive in nature.
- Where samples sizes permitted, statistical analysis was performed using (Student's) t-test or chi-squares' (i.e., between patients and caregivers on selected items).
- A type I error rate of 5% (alpha = 0.05) was applied to each individual hypothesis test.
- Unless otherwise specified, the statistical tests were not significant or could not be performed due to small cell sizes.

RESULTS

- Of the 145 individuals who responded to survey invitations, 101 (70%) individuals completed the survey questionnaire (68 caregivers, 33 patients).
- Approximately 60% resided in Canada.
- Just over half of the survey respondents were employed (52%).
- Approximately 60% of the US sample was white, and 62% were married (these demographics were not collected for Canadian participants).
- Caregivers reported that the mean age of their 68 sons with hemophilia A was 8.2 years (standard deviation [SD], 4.8), ranging from 1 to 18 years.*

Table 1. Respondent Characteristics

Characteristic	Caregivers ^a (n = 68)	Patients (n = 33)	Total Sample (N = 101)
Sex (derived), n (%)^b			
Male	12 (17.6)	33 (100.0)	45 (44.6)
Female	56 (82.4)	0 (0)	56 (55.4)
Age in years (derived)^c			
Mean (SD)	38.2 (7.6)	33.9 (12.2)	
Range	19-57	18-58	
Ethnicity, n (%)^d			
Asian	1 (3.8)	0 (0)	1 (2.7)
African American/black	2 (7.7)	0 (0)	2 (5.4)
Hispanic or Latino	8 (30.8)	1 (9.1)	9 (24.3)
White	13 (50.0)	9 (81.8)	22 (59.5)
Other	2 (7.7)	1 (9.1)	3 (8.1)
Missing	0	1	1
Education, n (%)			
Less than high school degree or equivalent (e.g., GED)	1 (1.5)	1 (3.0)	2 (2.0)
High school or equivalent (e.g., GED)	16 (23.5)	12 (36.4)	28 (27.7)
Technical school or associate's degree (2-year college degree)	11 (16.2)	5 (15.2)	16 (15.8)
College or university	35 (51.5)	12 (36.4)	47 (46.5)
Graduate or professional	5 (7.4)	3 (9.1)	8 (7.9)

GED = general equivalency diploma.

^a The caregiver column displays demographic characteristics about the caregivers themselves, rather than the patients for whom they care.

^b Administered only to caregiver respondents. Patients were presumed to be male.

^c Derived age based on year of birth.

^d Administered to US respondents only.

Note: "Missing" numbers are not included in the denominator for percentage calculations.

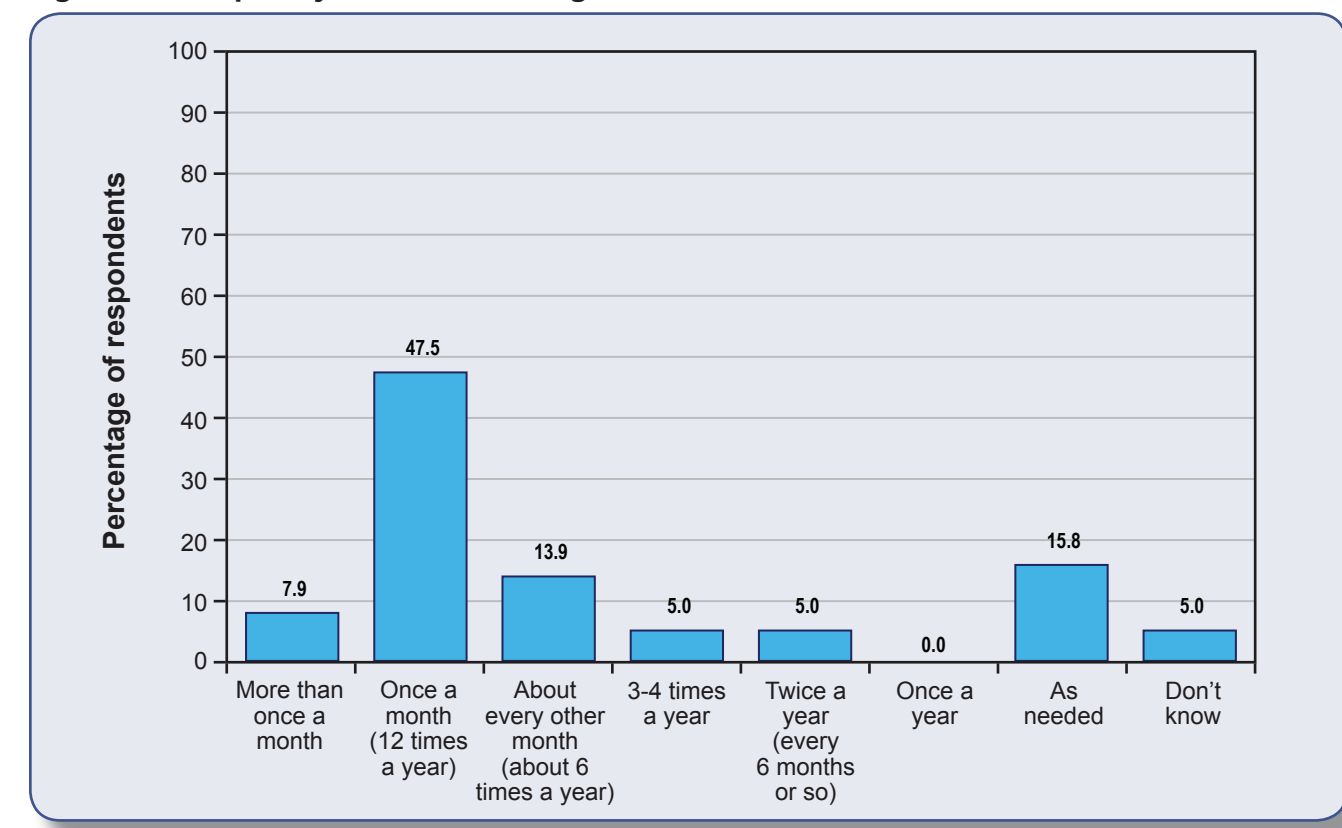
Clinical Characteristics and Product Usage

- More than two-thirds of the sample (69%) reported that they used their FVIII primarily as prophylaxis, and nearly one-quarter (24%) used it primarily on demand
- More than half of patients and caregivers (56%) reported infusing FVIII 3 days a week, followed by twice a week (21%) or 4 or more days a week (17%). Twenty-three percent reported using it less often per week or other (4%).
- Approximately 75% reported typically infusing single FVIII vials at any one time.

FVIII Ordering Behaviors

- Nearly half of survey respondents reported ordering FVIII monthly and nearly one-quarter ordered it less frequently (Figure 1).
- The mean number of FVIII concentrate vials in each order was 22 (SD = 21).

Figure 1. Frequency of FVIII Ordering



*While the caregiver survey was intended for those caring for children with hemophilia A who were younger than 18 years, caregivers were asked about their child's birth year rather than birth date. Thus, the age range extended to 18 years.

FVIII Storage Routines and Challenges

- More than half of the overall sample reported storing FVIII primarily in the refrigerator (55%), and 32% stored it at room temperature.
 - Patients and caregivers stored FVIII products differently (P < .05): patients reported a higher frequency of refrigerator storage than caregivers (61% vs. 42%, respectively), and a higher frequency of mixed room temperature and refrigerator storage (24% vs. 7%, respectively).
- Approximately one-third of respondents did not report any challenges with storing FVIII.
 - However, more than one-quarter reported that the need to plan for refrigeration when traveling for work/vacation (28%) and the need to carry an insulated tote bag when leaving the house (27%) were the biggest challenges to FVIII storage.
- While most respondents reported using FVIII vials prior to their expiration, nearly 8% of survey respondents indicated that they had FVIII vials that expired before they could be used.
 - Of these, 7 respondents (87.5%) reported using FVIII primarily on-demand and 1 (12.5%) as prophylaxis.

Table 2. FVIII Storage Routines and Challenges

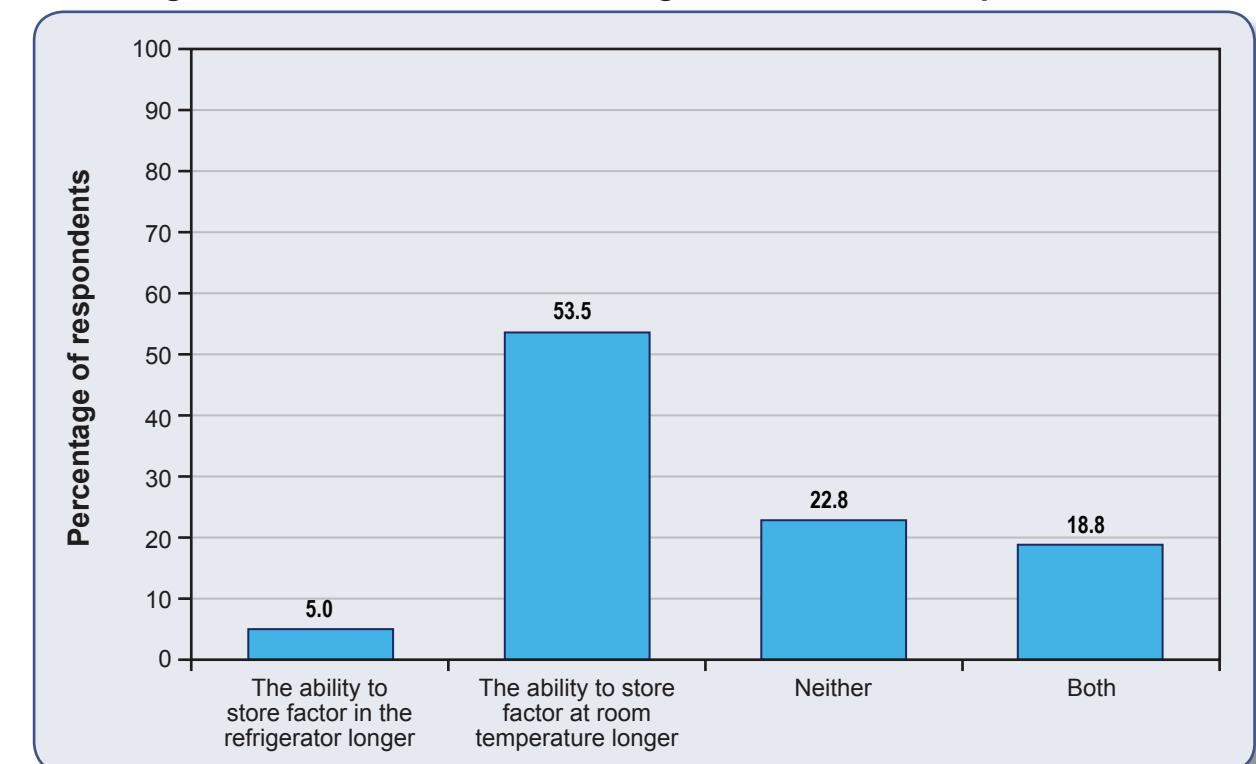
Characteristic	Total Sample (N = 101)
What are the biggest storage challenges, if any, associated with [your/your child's] current Factor VIII product? Select all that apply.	n (%)^a
The need to keep the factor refrigerated	19 (18.8)
The need to plan for refrigerating the factor when I travel for work or vacation	28 (27.7)
The length of time the factor can stay at room temperature	16 (15.8)
The length of time the factor can be refrigerated	4 (4.0)
The inability to return room temperature factor to the refrigerator	15 (14.9)
The inability to order a lot of factor at one time	19 (18.8)
The inability to store a lot of factor at one time	16 (15.8)
The length of time between mixing and using the factor	6 (5.9)
The need to carry an insulated tote bag for factor storage when I leave the house	27 (26.7)
None of the above	32 (31.7)

^a Column percentages do not sum to 100, because respondents were asked to select all options that apply.

FVIII Storage Preferences

- More than half (54%) of survey respondents noted that the ability to store a FVIII product at room temperature for longer duration was the most important attribute of a new FVIII product compared with the ability to store longer in the refrigerator (Figure 2).

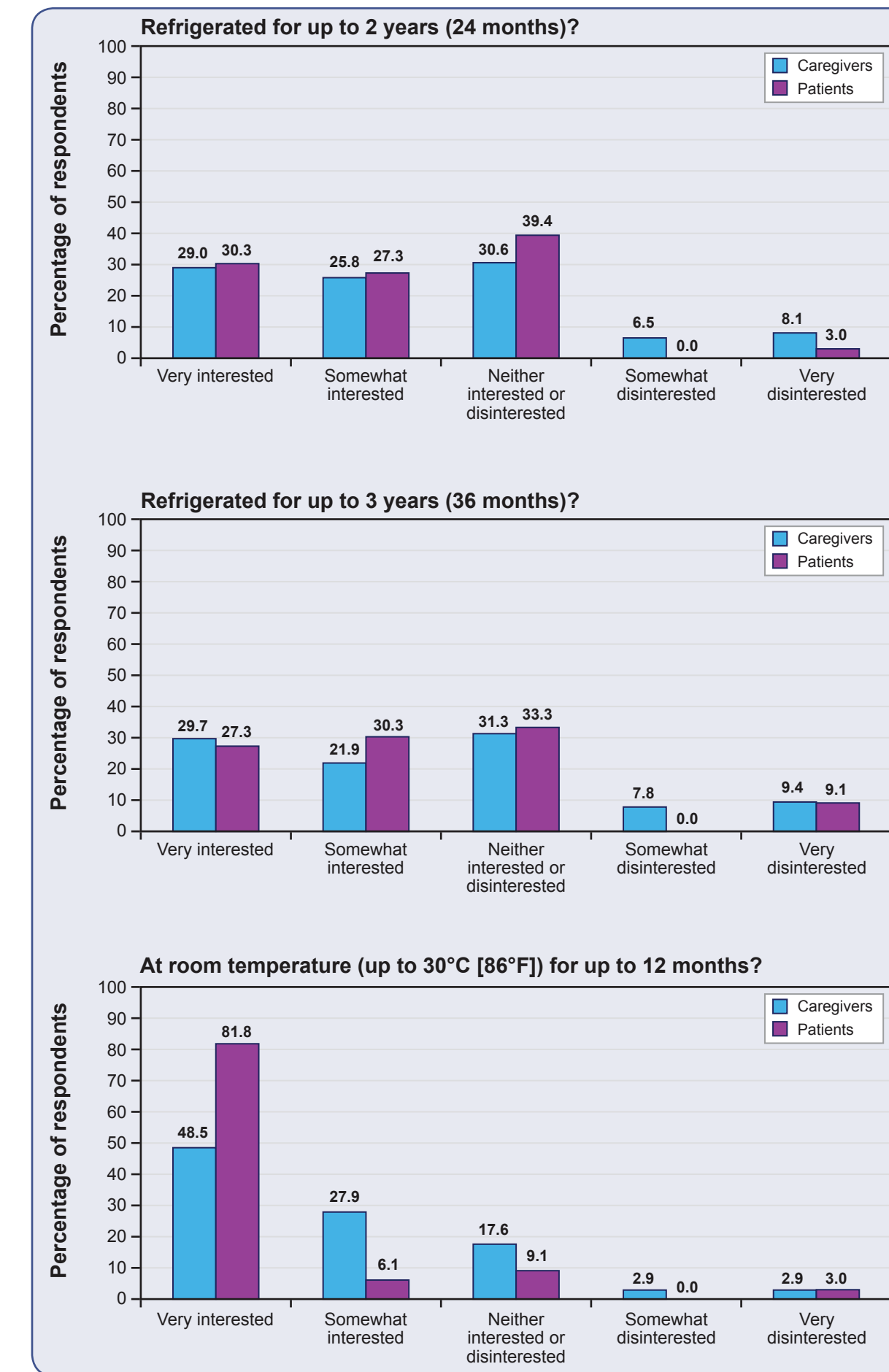
Figure 2. If a New Factor VIII Product Were Available, Which One of the Following Statements About Factor Storage Would Be Most Important to You?



- When asked about interest in a new FVIII product that worked just as well as their current FVIII but had different storage options, 80% of the overall sample indicated that they were very or somewhat interested in a FVIII product that could be stored at a higher room temperature for up to 12 months, compared with a FVIII product that could be refrigerated for up to 2 or 3 years (56% and 54%, respectively).

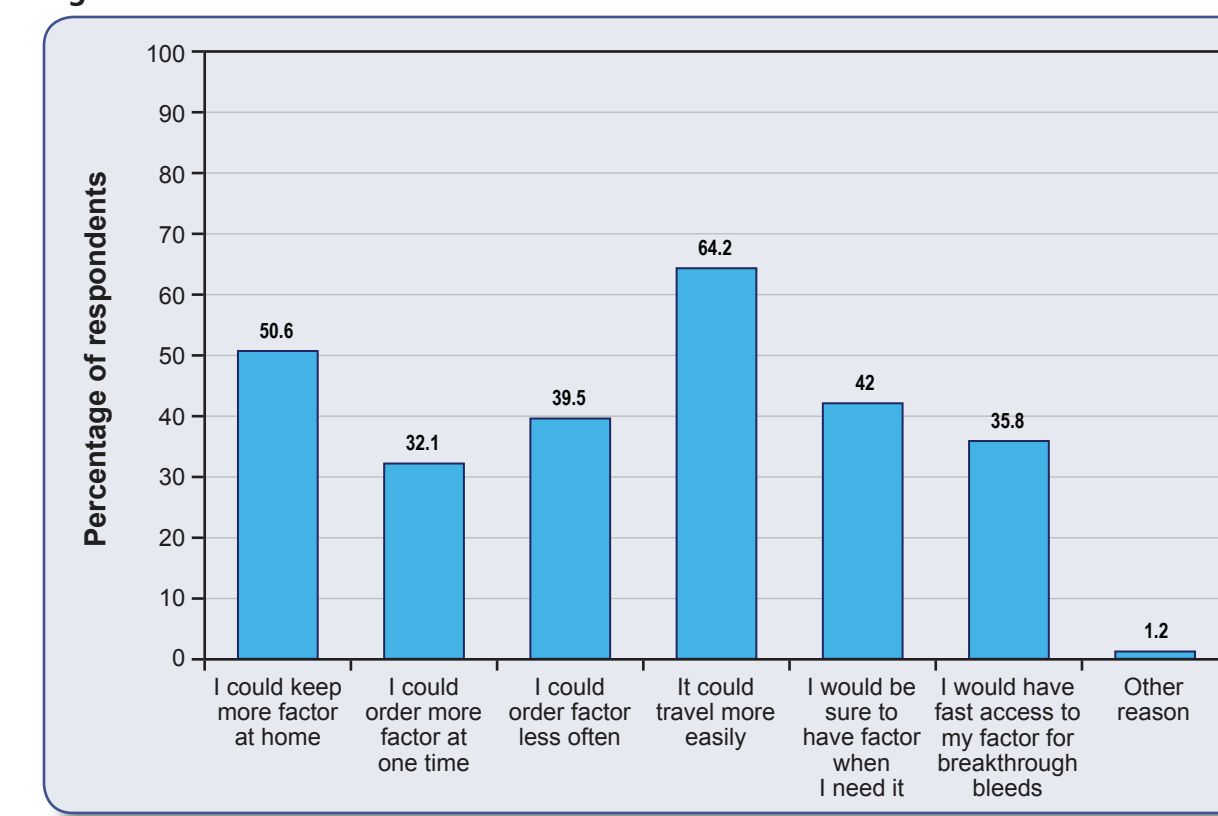
- A greater percentage of both patients and caregivers indicated a strong preference for storage at room temperature compared with the refrigeration options (Figure 3).

Figure 3. How Interested Would You Be in a New Factor VIII Product That Worked Just as Well as [Current FVIII] But That You Could Store...



- The primary reasons for interest in a new FVIII product that could be stored at a higher room temperature for up to 12 months included the ability to keep more factor at home (50.6%) and the ability to travel more easily (64%) (Figure 4).

Figure 4. Interest in a New FVIII Product



DISCUSSION

- Having a better understanding of patients' and caregivers experiences and perspective on hemophilia A treatments is important to disease management.
- FVIII storage remains a challenge, particularly with regard to travelling or even when leaving the house.
- Furthermore, a small percentage of respondents have had FVIII vials that expired before they could be used.
- Although the reasons for waste were not explored in the current study (e.g., ordering more FVIII than needed, not infusing as often), flexibility in FVIII storage options could potentially reduce the frequency of FVIII waste.
- Flexibility in FVIII storage is important to both patients and caregivers.
- A vast majority of both patients and caregivers expressed interest in a product that can be stored at a higher room temperature for longer durations (up to 30°C [86°F] for up to 12 months), primarily for:
 - Traveling more easily
 - Keeping more factor at home
 - Having easy access when needed

CONCLUSIONS

- This Web-based study provides a real-world perspective from both patients and caregivers on FVIII attributes that are important to this population.
- Results support the preference for a FVIII product that could potentially offer more convenience and faster access to patients in daily life and while traveling.
- Flexibility in storage potentially could reduce the frequency of FVIII waste.

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CONTACT INFORMATION

Dana Britt DiBenedetti, PhD
Head, Patient-Reported Outcomes

RTI Health Solutions
200 Park Offices Drive
Research Triangle Park, NC 27709

Phone: +1.919.316.3764
Fax: +1.919.541.7222
E-mail: ddbenedetti@rti.org

Presented at: ISPOR 18th Annual International Meeting
May 18-22, 2013
New Orleans, LA, United States

FUNDING

Funding for this study and development of this poster provided by Novo Nordisk A/S.

