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Treatment Preference of Moderate or Severe Hemophilia A Patients in Taiwan

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INTRODUCTION

- Hemophilia A is a bleeding disorder caused by the deficiency of factor VIII (FVIII). Multiple treatment paradigms exist for treated Hemophilia A patients. An understanding of patient treatment preferences is critical for enhancing adherence to treatment, identifying appropriate therapy for patients and maximizing health outcomes.
- This study was conducted to assess how patients and caregivers value different features of treatment when considering hemophilia treatment.

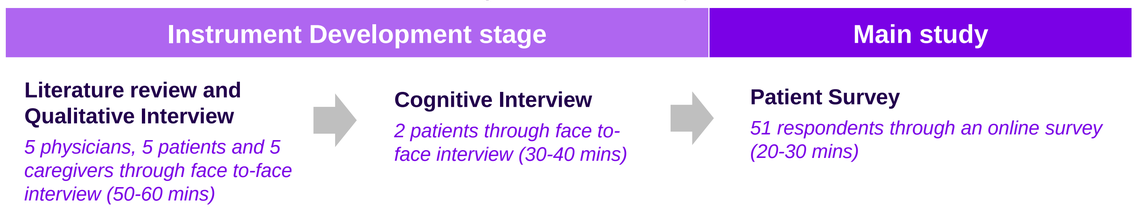
OBJECTIVES

- To Investigate treatment preference among patients and caregivers for Hemophilia A in Taiwan.
- To Understand the demographic and clinical characteristics of patients with Hemophilia A in Taiwan.

METHODS

- This is a single country, multicenter, cross-sectional, observational study.
- An online discrete choice experiment (DCE) survey was conducted between April 2021-April 2022 with a total of 51 respondents in Taiwan.
- Prior to DCE survey, the instrument development stage was designed to ensure the quality and clarity of survey instruments.
  - Literature review and exploratory qualitative interview were conducted to identify important treatment attributes and levels of attributes that were relevant to patients, which could be traded by DCE in this study.
  - The cognitive interview was then conducted after attributes were determined from insights of literature review and qualitative interview. The goal of this stage was to ensure the clarity of the content of the instrument in the population of interest and to refine the content as appropriate to be used in main study.

Figure 1. Overall study flow



Main study (DCE survey)

- A DCE approach was designed to assess respondents' willingness to accept trade-offs among hypothetical treatment profiles and provides information on key factors that drive an individual's choice.
- The respondent was asked to choose which of the options they would most prefer between two presented treatment profiles or neither one.
  - Assuming all other information not explicitly mentioned in the profiles is the same across treatments
  - Choice data were analyzed using Hierarchical Bayesian logistic regression model. Relative importance (RI) was calculated based on the differences of utility scores to determine the treatment preferences.

Figure 2. Example of Choice Task

Treatment A	Treatment B
No bleeding episode in ***45%*** of patients during 8 months; No bleeding episode in ***39%*** of patients during 3 years.	No bleeding episode in ***73%*** of patients in half a year, but lack of sufficient long-term efficacy data.
Averaged observed ABR for joint bleeding is ***1*** episode.	Averaged observed ABR for joint bleeding is ***2*** episodes.
Long-term safety has been proven through clinical trials and studies.	Lack of sufficient long-term safety data.
Factor VIII product. When breakthrough bleeding occur, could inject higher dose directly and there is ***no risk of thromboembolic event***. The medicine ***can use as on-demand therapy*** during bleeding event.	Non-factor VIII product. When breakthrough bleeding occurred, ***need extra high-dose Factor VIII*** for treatment and ***there is a risk of thromboembolic events***.
Prophylaxis treatment with ***3-5 days dosing frequency***. Frequency can be modified with lifestyle arrangement.	Prophylaxis treatment ***with 3 types of dosing frequency*** (once weekly, once every 2 weeks, or every 4 weeks). Frequency can't be modified with lifestyle arrangement.
Intravenous injection (IV).	Subcutaneous injection (SC).
rFcVIII pharmacokinetic can be monitored effectively.	Could only monitored drug concentration, but not rFcVIII pharmacokinetic data.
☐	☐
☐ Neither of the above	

- Study population inclusion criteria:

Hemophilia A patients

(a) Aged over 20 years; (b) Disease severity was moderate (clotting factor level was 1-2% and at least with one bleeding record) or severe (clotting factor level was <1%); (c) Could read and understand one of the main country languages; (d) Agree to answer the survey after signing an informed consent form.

Caregiver taking care of hemophilia A patients under 20 years old:

(a) Taking care of patient with hemophilia A patients whose disease severity was moderate (clotting factor level was 1-2% and at least with one bleeding record) or severe (clotting factor level was <1%).; (b) Could read and understand one of the main country languages; (c) Agree to answer the survey after signing an informed consent form.

RESULTS

Patient and caregiver profiles

- Among 51 study respondents recruited, 76% (n=39) were patients themselves. (Table 1)

Hemophilia patients (Table 1)

- Mostly were males (98%), 71% had severe Hemophilia A and 29% had moderate Hemophilia A. 43% had college degree and 29% were aged 20-29 years.
- 43% patients received prophylactic treatment for over 10 years, while 14% reported not having received prophylactic treatment.

Caregivers (Table 2)

- Half of them were males, and 42% aged 40-49 years.

Table 1. Respondent's profile (N=51)

		N	%
Disease severity	Moderate	15	29%
	Severe	36	71%
Years of receiving prophylactic treatment	Did not received prophylactic treatment	7	14%
	Less than 1 year	0	0%
	1 to 5 years	12	24%
	6 to 10 years	10	20%
	Over 10 years	22	43%
Gender	Male	50	98%
	Female	1	2%
Age	0 to 9 years old	4	8%
	10 to 19 years old	8	16%
	20 to 29 years old	15	29%
	30 to 39 years old	7	14%
	40 to 49 years old	6	12%
	50 to 59 years old	8	16%
	60 to 69 years old	2	4%
	70 to 79 years old	1	2%
	Over 80 years old	0	0%
Education level	Junior High School or lower	8	16%
	Senior high school	16	31%
	College/university	22	43%
	Master's degree	5	10%
	Doctoral degree	0	0%

Table 2. Caregiver's profile (N=12)

		N	%
Gender	Male	6	50%
	Female	6	50%
Age	20 to 29 years old	1	8%
	30 to 39 years old	4	33%
	40 to 49 years old	5	42%
	50 to 59 years old	1	8%
	60 to 69 years old	1	8%

Treatment Preference

- A total of 7 attributes were selected into DCE model with 2 different levels in each attribute.
- Type of treatment and risk of thromboembolic events (RI = 26.2%) was the most important attribute followed by consumption route (RI = 25.8%) and administration frequency (RI = 15.2%). Monitoring dosing options (RI = 6.3%) were not as important compared to the other attributes. (Figure 3)
- Within "treatment type and risk of thromboembolic events", respondents preferred "Factor VIII product with no risk of thromboembolic events" over "non-Factor VIII product with risk of thromboembolic events". (Table 3)

Figure 3. Relative Importance

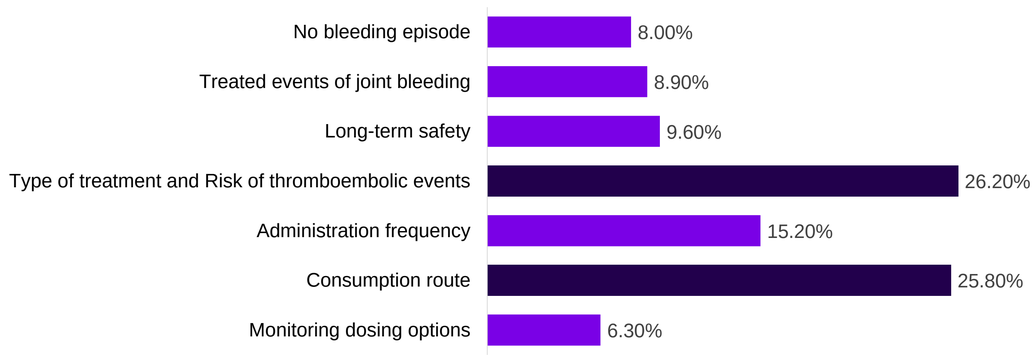


Table 3. Attribute-level preference weights (Utility)

Attribute	Levels	Utility	95% CI	
No bleeding episode	No bleeding episode in 45% of patients during 8 months; No bleeding episode in 39% of patients during 3 years.	-0.14	-0.26	-0.02
	No bleeding episode in 73% of patients in half a year, but lack of sufficient long-term efficacy data.	0.14	0.02	0.26
Treated events of joint bleeding	Averaged observed ABR for joint bleeding is 1 episode.	0.32	0.23	0.42
	Averaged observed ABR for joint bleeding is 2 episodes.	-0.32	-0.42	-0.23
Long-term safety	Long-term safety has been proven through clinical trials and studies.	0.29	0.14	0.45
	Lack of sufficient long-term safety data.	-0.29	-0.45	-0.14
Type of treatment and Risk of thromboembolic events	Factor VIII product. When breakthrough bleeding occur, could inject higher dose directly and there are no risk of thromboembolic events. The medicine can use as on-demand therapy during bleeding event.	1.18	0.89	1.47
	Non Factor VIII product. When breakthrough bleeding occur, need extra high-dose Factor VIII for treatment and there are risk of thromboembolic events.	-1.18	-1.47	-0.89
Administration frequency	Prophylaxis treatment with 3-5 days dosing frequency. Frequency can be modified with lifestyle arrangement.	-0.38	-0.60	-0.16
	Prophylaxis treatment with 3 types of dosing frequency (once weekly, once every 2 weeks, or every 4 weeks). Frequency can't be modified with lifestyle arrangement.	0.38	0.16	0.60
Consumption route	Intravenous injection (IV).	-0.68	-1.07	-0.29
	Subcutaneous injection (SC).	0.68	0.29	1.07
Monitoring dosing options	rFcVIII pharmacokinetic can be monitored effectively.	0.23	0.16	0.31
	Could only monitored drug concentration, but not rFcVIII pharmacokinetic data.	-0.23	-0.31	-0.16

CONCLUSION

- Taiwanese patients with hemophilia A valued "Type of treatment and Risk of thromboembolic events" and "Consumption route" as the top two important factors when considering a hemophilia therapy.

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