PREFERENCES FOR HEMOPHILIA TREATMENTS IN CANADA: A DISCRETE CHOICE EXPERIMENT (DCE)

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BACKGROUND

- Hemophilia A is a rare congenital disorder that is caused by a deficiency of factor VIII (FVIII) and results in bleeding episodes, bleeding-related complications, and disability.[1,2]
- The health-related quality of life (HRQoL) of patients with hemophilia is negatively affected by both the disease and treatment.[1]
- The main treatment goal is to prevent and treat bleeding, generally via administration of specific factor concentrate to compensate for the deficient clotting factor (FVIII).[2]
- Current treatments for hemophilia A in Canada include regular intravenous prophylactic FVIII infusions and on-demand treatment as bleeds occur.
- Given the burden of regular infusions on HRQoL, it has been noted that changes to treatment delivery may have positive impacts on treatment adherence, employment (economic), social and health aspects.[3]
- With the introduction of recently approved treatment options, including subcutaneous therapies such as emicizumab, understanding societal perspectives regarding treatment attributes is warranted.

OBJECTIVE

- The objective was to elicit preferences for features of hemophilia A treatments from a Canadian societal perspective.

METHODS

STUDY SAMPLE

- A convenience sample was recruited via market research panels and snowball sampling, with demographics intended to approximate the Canadian population.
- Participants included English-speaking adults living in Canada.
- This study was ethically approved by Veritas Independent Review Board (IRB; tracking Number: 16556-17:21:0124-04-2020); participants received $50 CAD compensation.

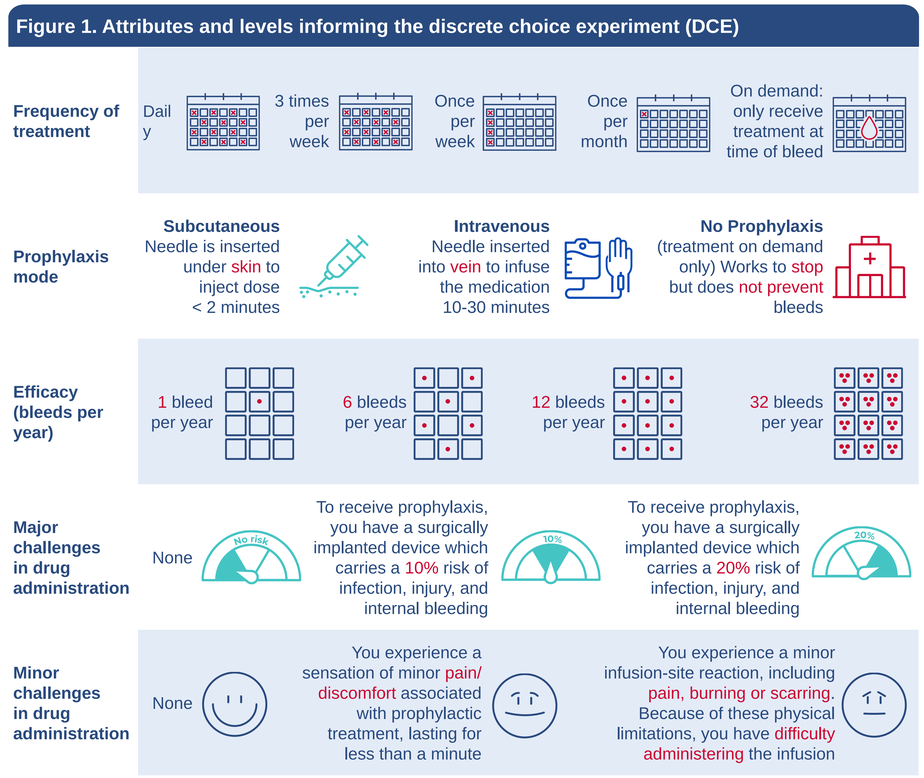
SURVEY DEVELOPMENT

- The survey and discrete choice experiment (DCE) attributes were designed based on a qualitative study conducted in n=10 individuals, including both adults with hemophilia A (n=2) and the caregivers of children with hemophilia (n=8).
- The elements included in the DCE survey, including the health endpoints, attributes, and levels of attributes of interest, were initially identified through a scoping review of the literature.

SURVEY ADMINISTRATION

- The DCE was administered in Canada via a self-directed online survey from August 2020 through September 2020, and included attributes and levels as described in **Figure 1.**

Figure 1. Attributes and levels informing the discrete choice experiment (DCE)



METHODS CONTINUED

- Participants were presented with a series of explanatory infographics, attribute descriptions, and graphical choice scenarios.
- Ten choice scenarios were presented in a randomized order and the ordering of questions and participant responses were recorded**.**

ANALYSIS

- Baseline characteristics were summarized using standard summary measures, and DCE results were analyzed using conditional logistic regression.
- Exploratory subgroup analyses of the DCE were conducted by prior treatment history, where preferences were estimated for those with prior intravenous injection experience, prior subcutaneous injection experience, and for those with experience in both.

RESULTS

-The DCE was completed by n=219 participants (mean age 44.7 years; 50.9% female).

-Thirteen percent of participants had experience with intravenous infusions, 10% with subcutaneous injections, and 6% with both.

-In the overall sample, there was a significant preference for treatments with fewer bleeds, lower risk of treatment complications, less frequent injections, subcutaneous treatments, and prophylaxis over on-demand.

-The odds ratio for preferring a subcutaneous prophylaxis vs. on-demand was 4.11 (p<0.001), and 3.61 (p=0.006) for intravenous prophylaxis vs. on-demand **(Table 1a).**

-Exploratory subgroup analyses suggested that preference for subcutaneous vs. intravenous may be more pronounced in subgroups with prior real-world experience with intravenous (**Table 1b**), subcutaneous therapies (**Table 1c**), or both (**Table 1d**); however, differences were not statistically significant.

LIMITATIONS

-While preferences in the DCE study reflect a societal perspective, given the general population sample for which they were elicited, these individuals do not have direct experience with hemophilia or its treatment. Thus, their understanding based on study materials may not fully reflect the reality of hemophilia, which could have influenced their selections. The potential difference in results for the subgroups with intravenous and/or subcutaneous therapies is reflective of this. Future research targeting larger groups of individuals with relevant experience would be of value to obtain more nuanced results.

CONCLUSIONS

-In Canadian general population respondents, preference was expressed for hemophilia A treatments having favorable efficacy and safety profiles, subcutaneous treatment, and less-frequent prophylaxis. Preference for subcutaneous over intravenous treatment modality warrants further investigation, and potential exploration among a patient, rather than general population, sample.

FUNDING AND DISCLOSURES

-This study was funded by Hoffmann-La Roche Limited, Canada. SO and DD are employees of Hoffmann-La Roche Limited, Canada, and honoraria was provided for consultation services to JMS and RJK.

Table 1. Discrete choice experiment (DCE) conditional logit regression results

Parameter	a. Overall study sample (n=219)			b. Intravenous treatment experienced (n=28)			c. Subcutaneous treatment experienced (n=22)			d. Subcutaneous and intravenous treatment experienced (n=14)		
	Estimate	P-value	Odds ratio	Estimate	P-value	Odds ratio	Estimate	P-value	Odds ratio	Estimate	P-value	Odds ratio
Efficacy (per additional bleed)	-0.029	<0.001	0.97	-0.017	0.270	0.98	-0.016	0.468	0.98	0.011	0.772	1.01
Major safety – 10% risk (relative to no risk)	0.390	0.179	1.48	-0.116	0.885	0.89	1.175	0.339	3.24	0.277	0.921	1.32
Major safety – 20% risk (relative to no risk)	-1.174	<0.001	0.31	-1.069	0.037	0.34	-1.179	0.056	0.31	-1.502	0.183	0.22
Minor safety – pain/discomfort (relative to no risk)	-1.094	<0.001	0.33	-1.335	0.066	0.26	-0.777	0.436	0.46	-2.086	0.347	0.12
Minor safety – infusion-site reaction (relative to no risk)	-1.189	<0.001	0.30	-0.919	0.082	0.40	-0.354	0.587	0.70	-0.077	0.947	0.93
IV prophylaxis (relative to on-demand)	1.282	0.006	3.61	1.546	0.237	4.69	-0.004	0.998	1.00	-0.680	0.840	0.51
Subcutaneous prophylaxis (relative to on-demand)	1.413	<0.001	4.11	2.138	0.049	8.48	0.040	0.754	1.50	0.698	0.755	2.01
Frequency (injections per week)	-0.158	<0.001	0.85	-0.110	0.252	0.90	-0.090	0.452	0.91	0.102	0.609	1.11

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