Impact of Dosing and Administration on HRQoL Among People With Hemophilia: A Time Trade-Off Study Including 2.250 Respondents in the UK, Canada and the US

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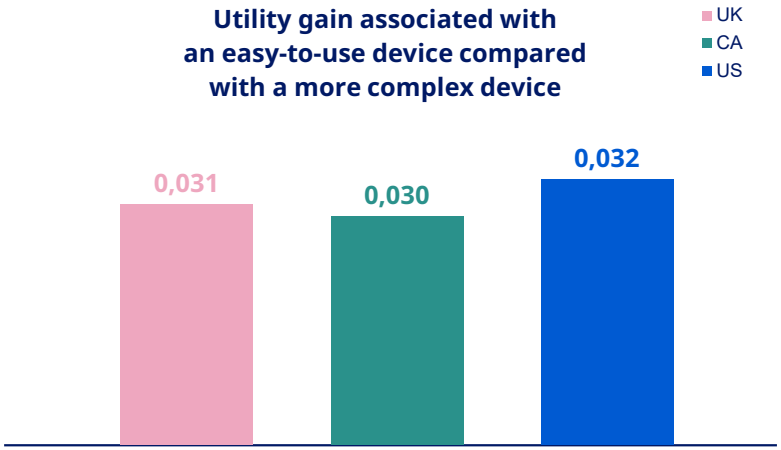
Utility gain associated with an easy-to-use device compared with a more complex device



Easy-to-use devices associated with fewer preparation steps and a short time-use may increase HRQoL among people living with hemophilia

BACKGROUND & AIMS

- Hemophilia is a rare hereditary bleeding disorder characterized by spontaneous bleedings due to deficiency or absence of clotting factor. The most common site for bleeds is the joints, and the bleedings can be painful and may restrict daily activities.
- Prophylactic treatments are administered as subcutaneous (SC) injections or intravenous (IV) infusions, both with varying treatment frequencies and complexities. Despite the benefits, previous studies have shown a high perceived treatment burden among people with hemophilia due to, e.g., a complicated and time-consuming preparation and administration process.[1-3]
- Treatment is lifelong, and it is therefore crucial to minimize treatment burden for people with hemophilia. The aim of this study was to investigate the impact from different treatment options on HRQoL.

MATERIAL & METHODS

- TTO methodology was used to estimate utilities through an online survey completed by the adult general (non-hemophilia) population in the UK, Canada (CA) and the US. Only males were included.
- To increase the relevance of results, both medical and health economic experts were included in the designing of the survey. In addition, data from a previous TTO study and focus group interviews including people with hemophilia was used (not published).
- Respondents evaluated health states as though they were living with hemophilia and treating themselves with prophylaxis.
- Respondents were excluded if they did not meet the inclusion criteria (male and 18+ years) or finish the survey (Table 1).
- The following aspects were evaluated: frequency, device complexity (preparation steps, administration and time-use) and injection site reactions (Table 2).

Table 1 Overview of respondents

	UK	CA	US
Respondents, n	1,171	1,104	1,601
Included in analysis, n (%)	812 (69)	739 (67)	703 (62)
Mean age, years	49.8	49.2	51.5

REFERENCES
1. Srivastava A, et al. Haemophilia. 2020 Aug, 26(56):1–158.
2. Brod M et al. Journal of Patient-Reported Outcomes. 2023 Feb, 23;7(1):17.
3. Thornburg C, et al. Patient Preference and Adherence. 2017 Sep; Vol 11:1677–86.

DISCLOSURES
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Table 2 Overview of health states included in the study

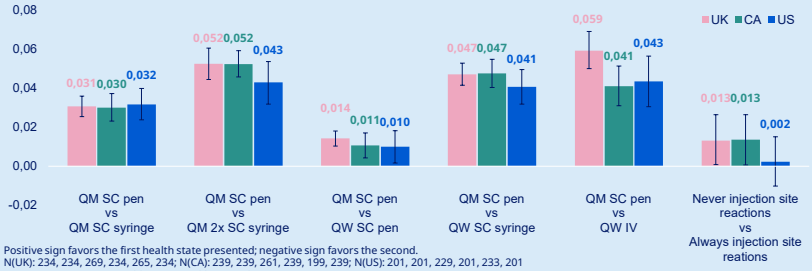
Health states							
Treatment device	SC pen	SC pen	SC syringe	SC syringe	SC syringe	2x SC syringe	IV
Frequency of treatment	QM	QW	QM	QM	QW	QM	QW
Injection site reactions	Never	Never	Always	Never	Never	Never	Never

IV: Intravenous; QM: Monthly; QW: Weekly; SC: Subcutaneous

RESULTS

- The results suggest that treatment device, including the complexity and time-use associated with preparation and administration, is a key factor.
- Using a pre-filled pen for SC injections once every month (QM) instead of a syringe was associated with a significant utility gain of 0.03 compared with QM SC injections with a syringe (p<0.001, 95% CI UK: 0.021-0.040, CA: 0.020-0.040, US: 0.019-0.044).
- QM SC injections with a pen were also associated with a significant utility gain compared with:
  1) QM 2x SC injections with a syringe (UK and CA: 0.05; US: 0.043 (p<0.001; 95% CI UK: 0.040-0.066, CA: 0.040-0.065, US: 0.030-0.056)).
  2) Weekly (QW) SC injections with a syringe (UK and CA: 0.05, US: 0.04 (p<0.001; 95% CI UK and CA: 0.036-0.059, US: 0.029-0.052)).
  3) QW IV infusions (UK: 0.6, CA and US: 0.4 (p<0.001; 95% CI UK: 0.049-0.070, CA: 0.026-0.056, US: 0.029-0.059)).
- A lower frequency was also preferred; QM instead of QW SC injections with a pen-device were associated with a significant utility gain of 0.01 (UK: p<0.001, 95% CI: 0.009-0.020; CA: p=0.005, 95% CI: 0.004-0.018; US: p=0.001, 95% CI: 0.002-0.018).
- Avoiding injection site reactions was associated with a significant utility gain of 0.01 in the UK and CA (p<0.001, 95% CI UK: 0.005-0.021, CA: 0.006-0.022). In the US, there were no significant results for this aspect.
- The overall preferences did not differ among the UK, CA and the US, and there were no significant differences in results among countries.

Figure 1 Utility gains or disutilities associated with different aspects of hemophilia treatment



Positive sign favors the first health state presented; negative sign favors the second.
N(UK): 234, 234, 269, 234, 265, 234; N(CA): 239, 239, 261, 239, 199, 239; N(US): 201, 201, 229, 201, 233, 201
IV: Intravenous; QM: Monthly; QW: Weekly; SC: Subcutaneous

CONCLUSIONS

- Based on answers from more than 2,250 respondents, this study finds that different aspects of hemophilia treatment have an impact.
- Future treatments should focus on easy-to-use devices that require few preparation steps and a short time-use. Additionally, the results indicate that less frequent treatments and treatments not associated with injection site reactions have the potential to decrease the treatment burden.
- The results emphasize the importance of individualizing treatment.
- In the future, the utility values found in this study can be used to estimate the value of different prophylactic treatments for hemophilia. Additionally, the values can be used in future cost-effectiveness analyses.