

Study Suggests Patients' Preferences Evolving Along with Treatments

In recent years, the arrival of novel hemophilia treatments that are effective and less burdensome to administer have opened up new possibilities for the consumers of these therapies. This begs the question – How might this evolving landscape be impacting treatment preferences for people with hemophilia?

Investigators sought answers to this question in a new study, “Patient and Caregiver Preferences for Haemophilia Treatments: A Discrete-Choice Experiment,” which was published in the journal [Haemophilia](#). The study was informed by a literature review and a survey open to adult males with hemophilia age 18 years and older, and caregivers of teen/adult males with hemophilia age 17 years or younger.

The surveys, which were submitted online from February to April 2022, generated a sample of 151 affected adults and 151 caregiver respondents. Each respondent evaluated hypothetical hemophilia treatment profiles defined by six attributes via a discrete choice experiment (DCE). The DCE was used by the authors to quantify preferences and learn more about trade-offs individuals consider when making decisions about available treatments.

Respondents answered questions based on six attributes: number of annual spontaneous bleeds; ability to live a more active lifestyle; how a medicine is prepared/administered; frequency of administration; risk of an inhibitor; risk of hospitalization due to treatment side-effects.

The burdens of treatment administration (intravenous and subcutaneous) and storage were also explored through a best-worst (BWS) scaling exercise, used to assess an individuals' priorities. It captures extremes including best and worst items, most and least important factors, biggest and smallest influences.

“In the BWS exercise, adult respondents and caregivers had overall similar preferences regarding the burden of treatment administration features, reported the investigators “Both samples found frequent and longer IV infusions most burdensome and a Sub-Q injection every 2 months least burdensome.”

DCE results indicated that both adult patients and caregivers preferred treatments that enabled a more active lifestyle and are associated with a lower inhibitor risk. Notably, both groups valued an active life *more* than reducing spontaneous bleeds.

“These findings suggest that adults with haemophilia and caregivers of children with haemophilia are willing to make tradeoffs for potential improvements in lifestyle not offered by clotting factor concentrates, bypass agents or activated factor VIII mimetics,” explained the authors.

The paper also acknowledged limitations. The sample generated from the survey included English speakers exclusively and respondents were predominantly white, highly educated. In addition, the survey was only available online, which would exclude individuals/families who do not have internet.

The authors posit that this study underscores the importance of a shared decision-making (SDM) approach to hemophilia care. Through SDM, healthcare providers and patients/caregivers may arrive at decisions that factor in the current treatment landscape and individual preferences.

Citation

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